

Connecting via Winsock to STN

Welcome to STN International! Enter x:x

LOGINID:SSPTAEXO1623

PASSWORD:

* * * * * RECONNECTED TO STN INTERNATIONAL * * * * *
SESSION RESUMED IN FILE 'BIOSIS, EMBASE, MEDLINE' AT 16:29:03 ON 23 OCT 2006
FILE 'BIOSIS' ENTERED AT 16:29:03 ON 23 OCT 2006
Copyright (c) 2006 The Thomson Corporation
FILE 'EMBASE' ENTERED AT 16:29:03 ON 23 OCT 2006
Copyright (c) 2006 Elsevier B.V. All rights reserved.
FILE 'MEDLINE' ENTERED AT 16:29:03 ON 23 OCT 2006f

COST IN U.S. DOLLARS	SINCE FILE ENTRY	TOTAL SESSION
FULL ESTIMATED COST	23.48	25.52

=> file registry

COST IN U.S. DOLLARS	SINCE FILE ENTRY	TOTAL SESSION
FULL ESTIMATED COST	23.48	25.52

FILE 'REGISTRY' ENTERED AT 16:29:09 ON 23 OCT 2006
USE IS SUBJECT TO THE TERMS OF YOUR STN CUSTOMER AGREEMENT.
PLEASE SEE "HELP-USAGETERMS" FOR DETAILS.
COPYRIGHT (C) 2006 American Chemical Society (ACS)

Property values tagged with IC are from the ZIC/VINITI data file provided by InfoChem.

STRUCTURE FILE UPDATES: 22 OCT 2006 HIGHEST RN 911002-75-0
DICTIONARY FILE UPDATES: 22 OCT 2006 HIGHEST RN 911002-75-0

New CAS Information Use Policies, enter HELP USAGETERMS for details.

TSCA INFORMATION NOW CURRENT THROUGH June 30, 2006

Please note that search-term pricing does apply when conducting SmartSELECT searches.

REGISTRY includes numerically searchable data for experimental and predicted properties as well as tags indicating availability of experimental property data in the original document. For information on property searching in REGISTRY, refer to:

<http://www.cas.org/ONLINE/UG/regprops.html>

=> s clitocine/cn

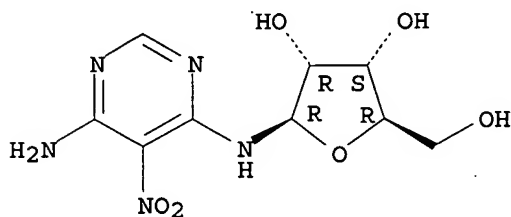
L8 1 CLITOCINE/CN

=> d l8

L8 ANSWER 1 OF 1 REGISTRY COPYRIGHT 2006 ACS on STN
RN 105798-74-1 REGISTRY
ED Entered STN: 21 Dec 1986
CN β -D-Ribofuranosylamine, N-(6-amino-5-nitro-4-pyrimidinyl)- (9CI) (CA INDEX NAME)
OTHER NAMES:
CN Clitocine

FS STEREOSEARCH
 MF C9 H13 N5 O6
 CI COM
 SR CA
 LC STN Files: AGRICOLA, BEILSTEIN*, BIOSIS, CA, CAPLUS, CASREACT, DDFU,
 DRUGU, IPA, MEDLINE, NAPRALERT, PROUSDDR, TOXCENTER, USPATFULL
 (*File contains numerically searchable property data)

Absolute stereochemistry.



PROPERTY DATA AVAILABLE IN THE 'PROP' FORMAT

19 REFERENCES IN FILE CA (1907 TO DATE)
 5 REFERENCES TO NON-SPECIFIC DERIVATIVES IN FILE CA
 19 REFERENCES IN FILE CAPLUS (1907 TO DATE)

=> file caplus
 COST IN U.S. DOLLARS
 FULL ESTIMATED COST

SINCE FILE	TOTAL
ENTRY	SESSION
7.10	32.62

FILE 'CAPLUS' ENTERED AT 16:29:35 ON 23 OCT 2006
 USE IS SUBJECT TO THE TERMS OF YOUR STN CUSTOMER AGREEMENT.
 PLEASE SEE "HELP USAGETERMS" FOR DETAILS.
 COPYRIGHT (C) 2006 AMERICAN CHEMICAL SOCIETY (ACS)

Copyright of the articles to which records in this database refer is held by the publishers listed in the PUBLISHER (PB) field (available for records published or updated in Chemical Abstracts after December 26, 1996), unless otherwise indicated in the original publications. The CA Lexicon is the copyrighted intellectual property of the American Chemical Society and is provided to assist you in searching databases on STN. Any dissemination, distribution, copying, or storing of this information, without the prior written consent of CAS, is strictly prohibited.

FILE COVERS 1907 - 23 Oct 2006 VOL 145 ISS 18
 FILE LAST UPDATED: 22 Oct 2006 (20061022/ED)

Effective October 17, 2005, revised CAS Information Use Policies apply. They are available for your review at:

<http://www.cas.org/infopolicy.html>

=> s 18
 L9 19 L8

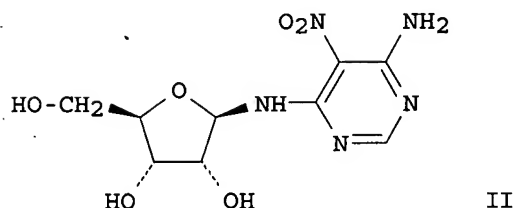
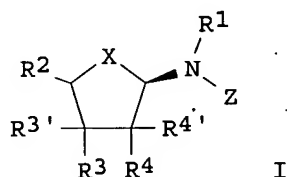
=> s 19 and ((muscular(w)dystrophy) or (cystic(w)fibrosis) or (nonsense(w)suppres?) or (premature(w)stop(w)codon))
 25752 MUSCULAR
 12657 DYSTROPHY
 8644 MUSCULAR(W)DYSTROPHY

15664 CYSTIC
 34783 FIBROSIS
 11864 CYSTIC(W) FIBROSIS
 7918 NONSENSE
 400106 SUPPRES?
 448 NONSENSE(W) SUPPRES?
 30766 PREMATURE
 39587 STOP
 37627 CODON

938 PREMATURE(W) STOP(W) CODON
 L10 2 L9 AND ((MUSCULAR(W) DYSTROPHY) OR (CYSTIC(W) FIBROSIS) OR (NONSENSE(W) SUPPRES?) OR (PREMATURE(W) STOP(W) CODON))

=> d l10 1-2 ti abs bib

L10 ANSWER 1 OF 2 CAPLUS COPYRIGHT 2006 ACS on STN
 TI Preparation of nucleoside analogs for treating or preventing diseases associated with nonsense mutations of mRNA
 GI



AB Nucleoside analogs I, wherein Z is (un)substituted alkyl, (un)substituted (un)substituted aryl, (un)substituted heteroaryl, (un)substituted cycloalkyl, (un)substituted heterocycle; X is CH, O, S, NH; R1 is H, (un)substituted alkyl, (un)substituted aryl, (un)substituted heteroaryl, (un)substituted cycloalkyl, (un)substituted heterocycle; R2 is (un)substituted alkyl, carboxy, amido, acyl, alkylcarbonyl, halogen, azide, alkyl amino, phosphate, phosphoester, alkyl ether; R3, R3', R4, R4' are independently (un)substituted ether, H, halogen, (un)substituted alkyl, (un)substituted (un)substituted aryl, (un)substituted heteroaryl, (un)substituted cycloalkyl, (un)substituted heterocycle are prepared for use in the treatment or prevention of diseases associated with nonsense mutations of mRNA. Thus, II was prepared and tested in a cell-based luciferase reporter assay containing a UGA premature termination codon that was stably transfected in 293T Human Embryonic Kidney cells (no data but very high potency and very high efficacy of protein synthesis). Further, I can be used as a prodrug in the treatment of autoimmune disease, blood diseases, collagen diseases, diabetes, neurodegenerative diseases, cardiovascular diseases, pulmonary diseases, inflammatory diseases, central nervous system diseases.

AN 2006:740594 CAPLUS
 DN 145:167496

TI Preparation of nucleoside analogs for treating or preventing diseases associated with nonsense mutations of mRNA
 IN Wilde, Richard G.; Almstead, Neil G.; Welch, Ellen M.; Beckmann, Holger
 PA USA

SO U.S. Pat. Appl. Publ., 47 pp.

CODEN: USXXCO

DT Patent

LA English

FAN.CNT 1

	PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI	US 2006166926	A1	20060727	US 2005-48659	20050121
PRAI	US 2005-48659		20050121		
OS	MARPAT 145:167496				

L10 ANSWER 2 OF 2 CAPLUS COPYRIGHT 2006 ACS on STN

TI Use of nucleoside compounds for nonsense suppression and the treatment of genetic diseases

AB The invention encompasses nucleoside compds., compns. comprising the compds. and methods for treating or preventing diseases associated with nonsense mutations of mRNA by administering these compds. or compns. Diseases that can be treated or prevented by compds. of the invention include, but are not limited to, cancer, autoimmune diseases, blood diseases, collagen diseases, diabetes, neurodegenerative diseases, cardiovascular diseases, pulmonary diseases, inflammatory diseases, lysosomal storage disease, tuberous sclerosis or central nervous system diseases. The present invention is based in part on the discovery of small mols. that modulate premature translation termination and/or nonsense-mediated mRNA decay.

AN 2004:80704 CAPLUS

DN 140:122839

TI Use of nucleoside compounds for nonsense suppression and the treatment of genetic diseases

IN Wilde, Richard G.; Almstead, Neil G.; Welch, Ellen M.; Beckmann, Holger

PA PTC Therapeutics, Inc., USA; Tularik Inc.

SO PCT Int. Appl., 93 pp.

CODEN: PIXXD2

DT Patent

LA English

FAN.CNT 2

	PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI	WO 2004009610	A2	20040129	WO 2003-US23185	20030723
	WO 2004009610	A3	20051006		
	W:				
	AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW				
	RW: GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW, AM, AZ, BY, KG, KZ, MD, RU, TJ, TM, AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR, BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG				
	CA 2493816	AA	20040129	CA 2003-2493816	20030723
	AU 2003261237	A1	20040209	AU 2003-261237	20030723
	EP 1572709	A2	20050914	EP 2003-766015	20030723
	EP 1572709	A3	20051123		
	R: AT, BE, CH, DE, DK, ES, FR, GB, GR, IT, LI, LU, NL, SE, MC, PT, IE, SI, LT, LV, FI, RO, MK, CY, AL, TR, BG, CZ, EE, HU, SK				
PRAI	US 2002-398334P	P	20020724		
	WO 2003-US23185	W	20030723		
OS	MARPAT 140:122839				

=> s 19 and (readthrough)

986 READTHROUGH

L11 1 L9 AND (READTHROUGH)

=> d l11 ti

L11 ANSWER 1 OF 1 CAPLUS COPYRIGHT 2006 ACS on STN
TI Use of nucleoside compounds for nonsense suppression and the treatment of genetic diseases

=> file uspatfull

COST IN U.S. DOLLARS

SINCE FILE

TOTAL

ENTRY

SESSION

FULL ESTIMATED COST

27.15

59.77

DISCOUNT AMOUNTS (FOR QUALIFYING ACCOUNTS)

SINCE FILE

TOTAL

ENTRY

SESSION

CA SUBSCRIBER PRICE

-1.50

-1.50

FILE 'USPATFULL' ENTERED AT 16:31:54 ON 23 OCT 2006

CA INDEXING COPYRIGHT (C) 2006 AMERICAN CHEMICAL SOCIETY (ACS)

FILE COVERS 1971 TO PATENT PUBLICATION DATE: 19 Oct 2006 (20061019/PD)

FILE LAST UPDATED: 19 Oct 2006 (20061019/ED)

HIGHEST GRANTED PATENT NUMBER: US7124445

HIGHEST APPLICATION PUBLICATION NUMBER: US2006236437

CA INDEXING IS CURRENT THROUGH 19 Oct 2006 (20061019/UPCA)

ISSUE CLASS FIELDS (/INCL) CURRENT THROUGH: 19 Oct 2006 (20061019/PD)

REVISED CLASS FIELDS (/NCL) LAST RELOADED: Jun 2006

USPTO MANUAL OF CLASSIFICATIONS THESAURUS ISSUE DATE: Jun 2006

=> s l8

L12 2 L8

=> s l12 and ((muscular(w)dystrophy) or (cystic(w)fibrosis) or (nonsense(w)suppres?) or (premature(w)stop(w)codon))

26657 MUSCULAR

8176 DYSTROPHY

5446 MUSCULAR(W)DYSTROPHY

15149 CYSTIC

24672 FIBROSIS

12495 CYSTIC(W)FIBROSIS

4379 NONSENSE

365999 SUPPRES?

112 NONSENSE(W)SUPPRES?

88242 PREMATURE

670811 STOP

53519 CODON

627 PREMATURE(W)STOP(W)CODON

L13 2 L12 AND ((MUSCULAR(W)DYSTROPHY) OR (CYSTIC(W)FIBROSIS) OR (NONSENSE(W)SUPPRES?) OR (PREMATURE(W)STOP(W)CODON))

=> d l13 1-2 ti

L13 ANSWER 1 OF 2 USPATFULL on STN

TI Use of nucleoside compounds for nonsense suppression and the treatment of genetic diseases

L13 ANSWER 2 OF 2 USPATFULL on STN

TI Nucleoside compounds and their use for treating cancer and diseases associated with somatic mutations

=> d l13 2 ti abs bib

L13 ANSWER 2 OF 2 USPATFULL on STN

TI Nucleoside compounds and their use for treating cancer and diseases

associated with somatic mutations
AB The invention encompasses nucleoside compounds, compositions comprising the compounds and methods for treating or preventing diseases associated with nonsense mutations of mRNA by administering these compounds or compositions.

CAS INDEXING IS AVAILABLE FOR THIS PATENT.

AN 2004:88924 USPATFULL
TI Nucleoside compounds and their use for treating cancer and diseases associated with somatic mutations
IN Wilde, Richard G., Somerville, NJ, UNITED STATES
Kennedy, Paul D., Kalamazoo, MI, UNITED STATES
Almstead, Neil G., Holmdel, NJ, UNITED STATES
Welch, Ellen M., Califon, NJ, UNITED STATES
Takasugi, James J., Lawrenceville, NJ, UNITED STATES
Friesen, Westley J., Huntingdon Valley, PA, UNITED STATES
PI US 2004067900 A1 20040408
AI US 2003-625059 A1 20030722 (10)
PRAI US 2002-398334P 20020724 (60)
DT Utility
FS APPLICATION
LREP JONES DAY, 222 EAST 41ST STREET, NEW YORK, NY, 10017
CLMN Number of Claims: 28
ECL Exemplary Claim: 1
DRWN 4 Drawing Page(s)
LN.CNT 3696

CAS INDEXING IS AVAILABLE FOR THIS PATENT.

=> index bioscience

FILE 'DRUGMONOG' ACCESS NOT AUTHORIZED

COST IN U.S. DOLLARS

SINCE FILE	TOTAL
ENTRY	SESSION
4.88	64.65

FULL ESTIMATED COST

DISCOUNT AMOUNTS (FOR QUALIFYING ACCOUNTS)

SINCE FILE	TOTAL
ENTRY	SESSION
0.00	-1.50

CA SUBSCRIBER PRICE

INDEX 'ADISCTI, ADISINSIGHT, ADISNEWS, AGRICOLA, ANABSTR, ANTE, AQUALINE, AQUASCI, BIOENG, BIOSIS, BIOTECHABS, BIOTECHDS, BIOTECHNO, CABA, CAPLUS, CEABA-VTB, CIN, CONFSCI, CROPB, CROPU, DDFB, DDFU, DGENE, DISSABS, DRUGB, DRUGMONOG2, DRUGU, EMBAL, EMBASE, ...' ENTERED AT 16:32:58 ON 23 OCT 2006

68 FILES IN THE FILE LIST IN STNINDEX

Enter SET DETAIL ON to see search term postings or to view search error messages that display as 0* with SET DETAIL OFF.

=> s. cliticine and ((nonsense(w)suppres?)or(readthrough) or(premature(w)stop))

26 FILES SEARCHED...

1 FILE IFIPAT

1 FILE PROUSDDR

56 FILES SEARCHED...

2 FILE USPATFULL

3 FILES HAVE ONE OR MORE ANSWERS, 68 FILES SEARCHED IN STNINDEX

L14 QUE CLITOCINE AND ((NONSENSE(W) SUPPRES?) OR (READTHROUGH) OR (PREMATURE(W) STOP))

=> d hid

DISPLAY L# IS NOT VALID IN STNINDEX

Answer set was created in a file. Enter DISPLAY HISTORY to see where the answer set was created. Use the File command to change to that

file, then display the answer.

=> d hid

DISPLAY L# IS NOT VALID IN STNINDEX

Answer set was created in a file. Enter DISPLAY HISTORY to see where the answer set was created. Use the File command to change to that file, then display the answer.

=> d his

(FILE 'HOME' ENTERED AT 15:10:21 ON 23 OCT 2006)

INDEX 'ADISCTI, ADISINSIGHT, ADISNEWS, AGRICOLA, ANABSTR, ANTE, AQUALINE, AQUASCI, BIOENG, BIOSIS, BIOTECHABS, BIOTECHDS, BIOTECHNO, CABA, CAPLUS, CEABA-VTB, CIN, CONFSCI, CROPB, CROPU, DDFB, DDFU, DGENE, DISSABS, DRUGB, DRUGMONOG2, DRUGU, EMBAL, EMBASE, ...' ENTERED AT 15:10:36 ON 23 OCT 2006
SEA FORSKOLIN AND (POTASSIUM(W) CHANNEL)

2 FILE AGRICOLA
1 FILE AQUASCI
1 FILE BIOENG
244 FILE BIOSIS
4 FILE BIOTECHABS
4 FILE BIOTECHDS
79 FILE BIOTECHNO
3 FILE CABA
402 FILE CAPLUS
24 FILE DDFU
5 FILE DISSABS
38 FILE DRUGU
1 FILE EMBAL
505 FILE EMBASE
48 FILE ESBIODBASE
35 FILE IFIPAT
1 FILE IMSRESEARCH
16 FILE JICST-EPLUS
14 FILE LIFESCI
189 FILE MEDLINE
21 FILE PASCAL
121 FILE SCISEARCH
151 FILE TOXCENTER
247 FILE USPATFULL
30 FILE USPAT2
1 FILE VETU
14 FILE WPIDS
14 FILE WPINDEX
L1 QUE FORSKOLIN AND (POTASSIUM(W) CHANNEL)

FILE 'BIOSIS, EMBASE, MEDLINE' ENTERED AT 15:12:06 ON 23 OCT 2006

L2 938 S FORSKOLIN AND (POTASSIUM(W) CHANNEL)
L3 182 S L2 AND (ATP(W) (SENSITIVE OR ACTIVATED))
L4 154 S L3 NOT PY>2003
L5 104 DUP REM L4 (50 DUPLICATES REMOVED)
L6 19 S L5 AND (ADENYLYL(W) CYCLASE)
L7 0 S L5 AND (BLOOD-BRAIN)

FILE 'REGISTRY' ENTERED AT 16:29:09 ON 23 OCT 2006

L8 1 S CLITOCINE/CN

FILE 'CAPLUS' ENTERED AT 16:29:35 ON 23 OCT 2006

L9 19 S L8
L10 2 S L9 AND ((MUSCULAR(W) DYSTROPHY) OR (CYSTIC(W) FIBROSIS) OR (NO
L11 1 S L9 AND (READTHROUGH)

FILE 'USPATFULL' ENTERED AT 16:31:54 ON 23 OCT 2006

L12 2 S L8

L13 2 S L12 AND ((MUSCULAR(W)DYSTROPHY) OR (CYSTIC(W)FIBROSIS) OR (NO

INDEX 'ADISCTI, ADISINSIGHT, ADISNEWS, AGRICOLA, ANABSTR, ANTE, AQUALINE, AQUASCI, BIOENG, BIOSIS, BIOTECHABS, BIOTECHDS, BIOTECHNO, CABA, CAPLUS, CEABA-VTB, CIN, CONFSCI, CROPB, CROPU, DDFB, DDFU, DGENE, DISSABS, DRUGB, DRUGMONOG2, DRUGU, EMBAL, EMBASE, ...' ENTERED AT 16:32:58 ON 23 OCT 2006
SEA CLITOCINE AND ((NONSENSE(W) SUPPRES?) OR (READTHROUGH) OR (PREM

1 FILE IFIPAT

1 FILE PROUSDDR

2 FILE USPATFULL

L14 QUE CLITOCINE AND ((NONSENSE(W) SUPPRES?) OR (READTHROUGH) OR (PR

=> logoff

ALL L# QUERIES AND ANSWER SETS ARE DELETED AT LOGOFF

LOGOFF? (Y)/N/HOLD:y

COST IN U.S. DOLLARS

SINCE FILE	TOTAL
ENTRY	SESSION
2.44	67.09

FULL ESTIMATED COST

DISCOUNT AMOUNTS (FOR QUALIFYING ACCOUNTS)

SINCE FILE	TOTAL
ENTRY	SESSION
0.00	-1.50

CA SUBSCRIBER PRICE

STN INTERNATIONAL LOGOFF AT 16:35:14 ON 23 OCT 2006

Connecting via Winsock to STN

Welcome to STN International! Enter x:x

LOGINID:SSPTAEXO1623

PASSWORD:

TERMINAL (ENTER 1, 2, 3, OR ?):2

* * * * * Welcome to STN International * * * * *

NEWS 1 Web Page URLs for STN Seminar Schedule - N. America
NEWS 2 "Ask CAS" for self-help around the clock
NEWS 3 AUG 09 INSPEC enhanced with 1898-1968 archive
NEWS 4 AUG 28 ADISCTI Reloaded and Enhanced
NEWS 5 AUG 30 CA(SM)/Caplus(SM) Austrian patent law changes
NEWS 6 SEP 11 CA/Caplus enhanced with more pre-1907 records
NEWS 7 SEP 21 CA/Caplus fields enhanced with simultaneous left and right
truncation
NEWS 8 SEP 25 CA(SM)/Caplus(SM) display of CA Lexicon enhanced
NEWS 9 SEP 25 CAS REGISTRY(SM) no longer includes Concord 3D coordinates
NEWS 10 SEP 25 CAS REGISTRY(SM) updated with amino acid codes for pyrrolysine
NEWS 11 SEP 28 CEABA-VTB classification code fields reloaded with new
classification scheme
NEWS 12 OCT 19 LOGOFF HOLD duration extended to 120 minutes
NEWS 13 OCT 19 E-mail format enhanced
NEWS 14 OCT 23 Option to turn off MARPAT highlighting enhancements available
NEWS 15 OCT 23 CAS Registry Number crossover limit increased to 300,000 in
multiple databases
NEWS 16 OCT 23 The Derwent World Patents Index suite of databases on STN
has been enhanced and reloaded

NEWS EXPRESS JUNE 30 CURRENT WINDOWS VERSION IS V8.01b, CURRENT
MACINTOSH VERSION IS V6.0c(ENG) AND V6.0Jc(JP),
AND CURRENT DISCOVER FILE IS DATED 26 JUNE 2006.

NEWS HOURS STN Operating Hours Plus Help Desk Availability
NEWS LOGIN Welcome Banner and News Items
NEWS IPC8 For general information regarding STN implementation of IPC 8
NEWS X25 X.25 communication option no longer available

Enter NEWS followed by the item number or name to see news on that
specific topic.

All use of STN is subject to the provisions of the STN Customer
agreement. Please note that this agreement limits use to scientific
research. Use for software development or design or implementation
of commercial gateways or other similar uses is prohibited and may
result in loss of user privileges and other penalties.

* * * * * STN Columbus * * * * *

FILE 'HOME' ENTERED AT 16:42:54 ON 23 OCT 2006

=> file registry

COST IN U.S. DOLLARS

SINCE FILE

TOTAL

ENTRY

SESSION

FULL ESTIMATED COST

0.21

0.21

FILE 'REGISTRY' ENTERED AT 16:43:05 ON 23 OCT 2006

USE IS SUBJECT TO THE TERMS OF YOUR STN CUSTOMER AGREEMENT.
PLEASE SEE "HELP USAGETERMS" FOR DETAILS.
COPYRIGHT (C) 2006 American Chemical Society (ACS)

Property values tagged with IC are from the ZIC/VINITI data file
provided by InfoChem.

STRUCTURE FILE UPDATES: 22 OCT 2006 HIGHEST RN 911002-75-0
DICTIONARY FILE UPDATES: 22 OCT 2006 HIGHEST RN 911002-75-0

New CAS Information Use Policies, enter HELP USAGETERMS for details.

TSCA INFORMATION NOW CURRENT THROUGH June 30, 2006

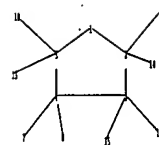
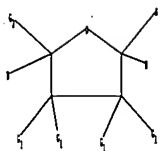
Please note that search-term pricing does apply when
conducting SmartSELECT searches.

REGISTRY includes numerically searchable data for experimental and
predicted properties as well as tags indicating availability of
experimental property data in the original document. For information
on property searching in REGISTRY, refer to:

<http://www.cas.org/ONLINE/UG/regprops.html>

=>

Uploading C:\Program Files\Stnexp\Queries\11048659generic.str



```

chain nodes :
7  8 10 11 12 13 14 15
ring nodes :
1  2 3 4 5
chain bonds :
2-12 2-14 3-11 3-15 4-7 4-8 5-10 5-13
ring bonds :
1-2 1-5 2-3 3-4 4-5
exact/norm bonds :
1-2 1-5 2-3 2-12 3-4 3-11 3-15 4-5 4-7 4-8 5-10
exact bonds :
2-14 5-13

```

G1:C,H,O

G2:C,O

Match level :

```

1:Atom 2:Atom 3:Atom 4:Atom 5:Atom 7:CLASS 8:CLASS 10:CLASS 11:CLASS
12:CLASS 13:CLASS 14:CLASS 15:CLASS

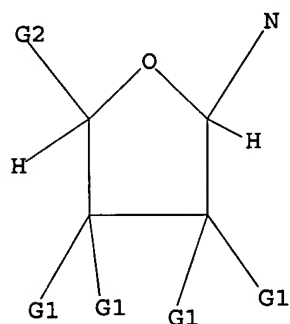
```

L1 STRUCTURE UPLOADED

=> d l1

L1 HAS NO ANSWERS

L1 STR



G1 C,H,O

G2 C,O

Structure attributes must be viewed using STN Express query preparation.

=> s l1 sss sam

SAMPLE SEARCH INITIATED 16:43:34 FILE 'REGISTRY'

SAMPLE SCREEN SEARCH COMPLETED - 13585 TO ITERATE

14.7% PROCESSED 2000 ITERATIONS

18 ANSWERS

INCOMPLETE SEARCH (SYSTEM LIMIT EXCEEDED)

SEARCH TIME: 00.00.01

FULL FILE PROJECTIONS: ONLINE **COMPLETE**
BATCH **COMPLETE**

PROJECTED ITERATIONS: 264718 TO 278682

PROJECTED ANSWERS: 1782 TO 3108

L2 18 SEA SSS SAM L1

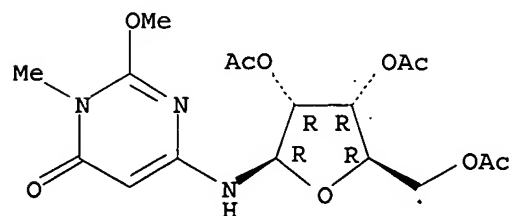
=> d l2 scan

L2 18 ANSWERS REGISTRY COPYRIGHT 2006 ACS on STN

IN 4(3H)-Pyrimidinone, 2-methoxy-3-methyl-6-[(2,3,5-tri-O-acetyl-β-D-ribofuranosyl)amino]- (9CI)

MF C17 H23 N3 O9

Absolute stereochemistry. Rotation (-).

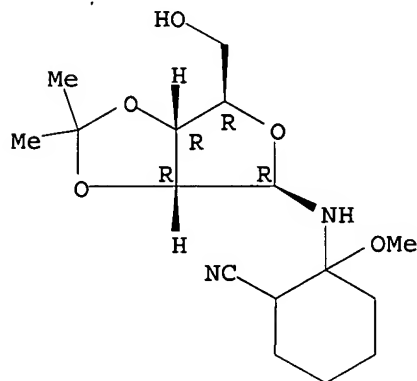


PROPERTY DATA AVAILABLE IN THE 'PROP' FORMAT

HOW MANY MORE ANSWERS DO YOU WISH TO SCAN? (1):4

L2 18 ANSWERS REGISTRY COPYRIGHT 2006 ACS on STN
IN Cyclohexanecarbonitrile, 2-methoxy-2-[[2,3-O-(1-methylethylidene)- β -D-ribofuranosyl]amino]- (9CI)
MF C16 H26 N2 O5

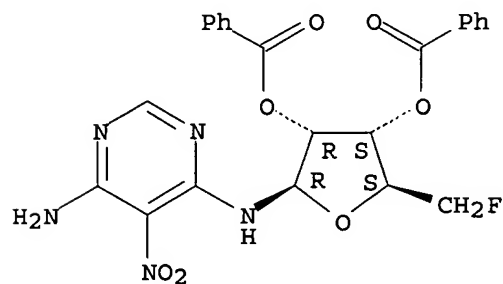
Absolute stereochemistry.



PROPERTY DATA AVAILABLE IN THE 'PROP' FORMAT

L2 18 ANSWERS REGISTRY COPYRIGHT 2006 ACS on STN
IN β -D-Ribofuranosylamine, N-(6-amino-5-nitro-4-pyrimidinyl)-5-deoxy-5-fluoro-, 2,3-dibenzoate (9CI)
MF C23 H20 F N5 O7

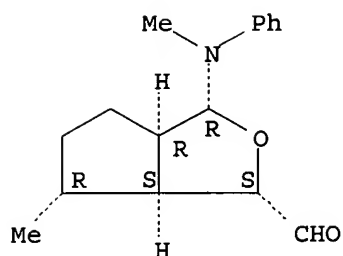
Absolute stereochemistry.



PROPERTY DATA AVAILABLE IN THE 'PROP' FORMAT

L2 18 ANSWERS REGISTRY COPYRIGHT 2006 ACS on STN
IN 1H-Cyclopenta[c]furan-1-carboxaldehyde, hexahydro-6-methyl-3-(methylphenylamino)-, (1 α ,3 α ,3 α ,6 α ,6 α)- (9CI)
MF C16 H21 N O2

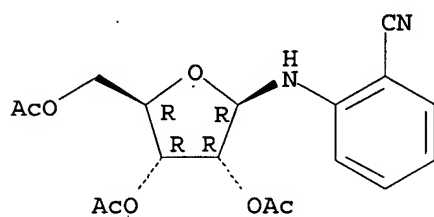
Relative stereochemistry.



PROPERTY DATA AVAILABLE IN THE 'PROP' FORMAT

L2 18 ANSWERS REGISTRY COPYRIGHT 2006 ACS on STN
 IN Benzonitrile, 2-[(2,3,5-tri-O-acetyl-β-D-ribofuranosyl)amino]- (9CI)
 MF C18 H20 N2 O7

Absolute stereochemistry.

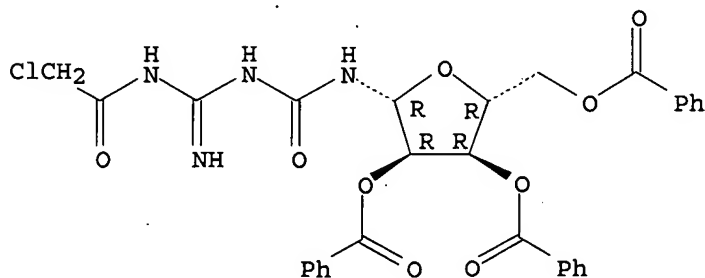


PROPERTY DATA AVAILABLE IN THE 'PROP' FORMAT

HOW MANY MORE ANSWERS DO YOU WISH TO SCAN? (1):4

L2 18 ANSWERS REGISTRY COPYRIGHT 2006 ACS on STN
 IN Acetamide, 2-chloro-N-[imino[[[(2,3,5-tri-O-benzoyl-β-D-ribofuranosyl)amino]carbonyl]amino]methyl]- (9CI)
 MF C30 H27 Cl N4 O9

Absolute stereochemistry.

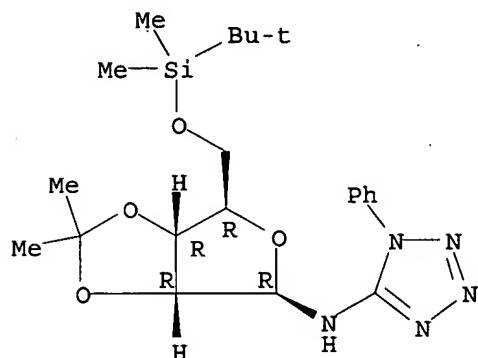


PROPERTY DATA AVAILABLE IN THE 'PROP' FORMAT

L2 18 ANSWERS REGISTRY COPYRIGHT 2006 ACS on STN
 IN β-D-Ribofuranosylamine, 5-O-[(1,1-dimethylethyl)dimethylsilyl]-2,3-O-

(1-methylethylidene)-N-(1-phenyl-1H-tetrazol-5-yl)- (9CI)
 MF C21 H33 N5 O4 Si

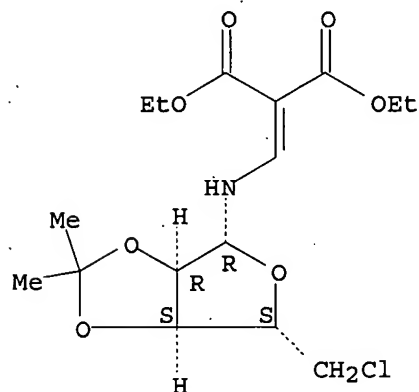
Absolute stereochemistry.



PROPERTY DATA AVAILABLE IN THE 'PROP' FORMAT

L2 18 ANSWERS REGISTRY COPYRIGHT 2006 ACS on STN
 IN Propanedioic acid, [[[5-chloro-5-deoxy-2,3-O-(1-methylethylidene)-β-D-ribofuranosyl]amino]methylene]-, diethyl ester (9CI)
 MF C16 H24 Cl N O7

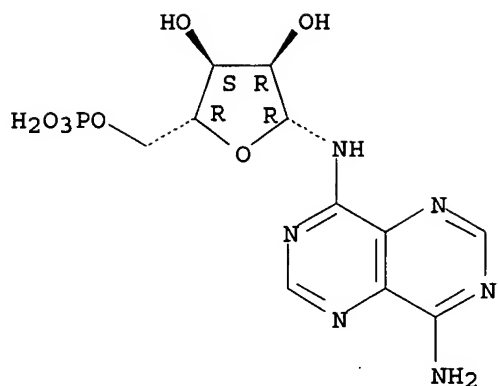
Absolute stereochemistry. Rotation (-).



PROPERTY DATA AVAILABLE IN THE 'PROP' FORMAT

L2 18 ANSWERS REGISTRY COPYRIGHT 2006 ACS on STN
 IN β-D-Ribofuranosylamine, N-(8-aminopyrimido[5,4-d]pyrimidin-4-yl)-, 5-(dihydrogen phosphate), monosodium salt (9CI)
 MF C11 H15 N6 O7 P . Na

Absolute stereochemistry.



● Na

HOW MANY MORE ANSWERS DO YOU WISH TO SCAN? (1):0

=> s l1 sss full

FULL SEARCH INITIATED 16:44:24 FILE 'REGISTRY'

FULL SCREEN SEARCH COMPLETED - 274489 TO ITERATE

100.0% PROCESSED 274489 ITERATIONS

2501 ANSWERS

SEARCH TIME: 00.00.02

L3 2501 SEA SSS FUL L1

=> file caplus

COST IN U.S. DOLLARS

SINCE FILE

TOTAL

ENTRY

SESSION

FULL ESTIMATED COST

167.38

167.59

FILE 'CAPLUS' ENTERED AT 16:44:30 ON 23 OCT 2006

USE IS SUBJECT TO THE TERMS OF YOUR STN CUSTOMER AGREEMENT.

PLEASE SEE "HELP USAGETERMS" FOR DETAILS.

COPYRIGHT (C) 2006 AMERICAN CHEMICAL SOCIETY (ACS)

Copyright of the articles to which records in this database refer is held by the publishers listed in the PUBLISHER (PB) field (available for records published or updated in Chemical Abstracts after December 26, 1996), unless otherwise indicated in the original publications. The CA Lexicon is the copyrighted intellectual property of the American Chemical Society and is provided to assist you in searching databases on STN. Any dissemination, distribution, copying, or storing of this information, without the prior written consent of CAS, is strictly prohibited.

FILE COVERS 1907 - 23 Oct 2006 VOL 145 ISS 18

FILE LAST UPDATED: 22 Oct 2006 (20061022/ED)

Effective October 17, 2005, revised CAS Information Use Policies apply. They are available for your review at:

<http://www.cas.org/infopolicy.html>

=> s l3

L4 1317 L3

=> s 14 and (nonsense(w) suppres?)

7918 NONSENSE

400106 SUPPRES?

448 NONSENSE(W) SUPPRES?

L5 1 L4 AND (NONSENSE(W) SUPPRES?)

=> d 15 1 ti

L5 ANSWER 1 OF 1 CAPLUS COPYRIGHT 2006 ACS on STN

TI Use of nucleoside compounds for nonsense suppression
and the treatment of genetic diseases

=> d 15 1 ti abs bib

L5 ANSWER 1 OF 1 CAPLUS COPYRIGHT 2006 ACS on STN

TI Use of nucleoside compounds for nonsense suppression
and the treatment of genetic diseases

AB The invention encompasses nucleoside compds., compns. comprising the
compds. and methods for treating or preventing diseases associated with
nonsense mutations of mRNA by administering these compds. or compns.
Diseases that can be treated or prevented by compds. of the invention
include, but are not limited to, cancer, autoimmune diseases, blood
diseases, collagen diseases, diabetes, neurodegenerative diseases,
cardiovascular diseases, pulmonary diseases, inflammatory diseases,
lysosomal storage disease, tuberous sclerosis or central nervous system
diseases. The present invention is based in part on the discovery of
small mols. that modulate premature translation termination and/or
nonsense-mediated mRNA decay.

AN 2004:80704 CAPLUS

DN 140:122839

TI Use of nucleoside compounds for nonsense suppression
and the treatment of genetic diseases

IN Wilde, Richard G.; Almstead, Neil G.; Welch, Ellen M.; Beckmann, Holger

PA PTC Therapeutics, Inc., USA; Tularik Inc.

SO PCT Int. Appl., 93 pp.

CODEN: PIXXD2

DT Patent

LA English

FAN.CNT 2

	PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI	WO 2004009610	A2	20040129	WO 2003-US23185	20030723
	WO 2004009610	A3	20051006		
	W:	AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW			
	RW:	GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW, AM, AZ, BY, KG, KZ, MD, RU, TJ, TM, AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR, BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG			
	CA 2493816	AA	20040129	CA 2003-2493816	20030723
	AU 2003261237	A1	20040209	AU 2003-261237	20030723
	EP 1572709	A2	20050914	EP 2003-766015	20030723
	EP 1572709	A3	20051123		
	R:	AT, BE, CH, DE, DK, ES, FR, GB, GR, IT, LI, LU, NL, SE, MC, PT, IE, SI, LT, LV, FI, RO, MK, CY, AL, TR, BG, CZ, EE, HU, SK			
PRAI	US 2002-398334P	P	20020724		
	WO 2003-US23185	W	20030723		
OS	MARPAT 140:122839				

=> file uspatfull
COST IN U.S. DOLLARS
FULL ESTIMATED COST

SINCE FILE	TOTAL
ENTRY	SESSION
7.43	175.02

DISCOUNT AMOUNTS (FOR QUALIFYING ACCOUNTS)
CA SUBSCRIBER PRICE

SINCE FILE	TOTAL
ENTRY	SESSION
-0.75	-0.75

FILE 'USPATFULL' ENTERED AT 16:45:11 ON 23 OCT 2006
CA INDEXING COPYRIGHT (C) 2006 AMERICAN CHEMICAL SOCIETY (ACS)

FILE COVERS 1971 TO PATENT PUBLICATION DATE: 19 Oct 2006 (20061019/PD)
FILE LAST UPDATED: 19 Oct 2006 (20061019/ED)
HIGHEST GRANTED PATENT NUMBER: US7124445
HIGHEST APPLICATION PUBLICATION NUMBER: US2006236437
CA INDEXING IS CURRENT THROUGH 19 Oct 2006 (20061019/UPCA)
ISSUE CLASS FIELDS (/INCL) CURRENT THROUGH: 19 Oct 2006 (20061019/PD)
REVISED CLASS FIELDS (/NCL) LAST RELOADED: Jun 2006
USPTO MANUAL OF CLASSIFICATIONS THESAURUS ISSUE DATE: Jun 2006

=> s 13
L6

58 L3

=> s 16 and (nonsnese(w)suppres?)

1 NONSNESE

365999 SUPPRES?

0 NONSNESE (W) SUPPRES?

L7

0 L6 AND (NONSNESE (W) SUPPRES?)

=> s 16 and (nonsnese(w)suppres?)

0 NONSENSE

365999 SUPPRES?

0 NONSENSE (W) SUPPRES?

L8

0 L6 AND (NONSENSE (W) SUPPRES?)

=> s 16 and (nonsense(w)suppres?)

4379 NONSENSE

365999 SUPPRES?

112 NONSENSE (W) SUPPRES?

L9

2 L6 AND (NONSENSE (W) SUPPRES?)

=> d 19 1-2 ti

L9 ANSWER 1 OF 2 USPATFULL on STN

TI Use of nucleoside compounds for nonsense suppression
and the treatment of genetic diseases

L9 ANSWER 2 OF 2 USPATFULL on STN

TI Nucleoside compounds and their use for treating cancer and diseases
associated with somatic mutations

=> file caplus
COST IN U.S. DOLLARS
FULL ESTIMATED COST

SINCE FILE	TOTAL
ENTRY	SESSION
51.45	226.47

DISCOUNT AMOUNTS (FOR QUALIFYING ACCOUNTS)
CA SUBSCRIBER PRICE

SINCE FILE	TOTAL
ENTRY	SESSION
0.00	-0.75

FILE 'CAPLUS' ENTERED AT 16:45:56 ON 23 OCT 2006
USE IS SUBJECT TO THE TERMS OF YOUR STN CUSTOMER AGREEMENT.

PLEASE SEE "HELP USAGETERMS" FOR DETAILS.
COPYRIGHT (C) 2006 AMERICAN CHEMICAL SOCIETY (ACS)

Copyright of the articles to which records in this database refer is held by the publishers listed in the PUBLISHER (PB) field (available for records published or updated in Chemical Abstracts after December 26, 1996), unless otherwise indicated in the original publications. The CA Lexicon is the copyrighted intellectual property of the American Chemical Society and is provided to assist you in searching databases on STN. Any dissemination, distribution, copying, or storing of this information, without the prior written consent of CAS, is strictly prohibited.

FILE COVERS 1907 - 23 Oct 2006 VOL 145 ISS 18
FILE LAST UPDATED: 22 Oct 2006 (20061022/ED)

Effective October 17, 2005, revised CAS Information Use Policies apply. They are available for your review at:

<http://www.cas.org/infopolicy.html>

=> s l4 and ((muscular(w)dystrophy) or readthrough or (cystic(w)fibrosis) or glaucoma or (genetic(w)(disease or disorder)))

25752 MUSCULAR

12657 DYSTROPHY

8644 MUSCULAR(W) DYSTROPHY

986 READTHROUGH

15664 CYSTIC

34783 FIBROSIS

11864 CYSTIC(W) FIBROSIS

7189 GLAUCOMA

745701 GENETIC

896125 DISEASE

252024 DISORDER

4118 GENETIC(W) (DISEASE OR DISORDER)

L10 4 L4 AND ((MUSCULAR(W)DYSTROPHY) OR READTHROUGH OR (CYSTIC(W)FIBROSIS) OR GLAUCOMA OR (GENETIC(W) (DISEASE OR DISORDER)))

=> d l10 1-4 ti

L10 ANSWER 1 OF 4 CAPLUS COPYRIGHT 2006 ACS on STN

TI Preparation of nucleoside analogs for treating or preventing diseases associated with nonsense mutations of mRNA

L10 ANSWER 2 OF 4 CAPLUS COPYRIGHT 2006 ACS on STN

TI Use of nucleoside compounds for nonsense suppression and the treatment of genetic diseases

L10 ANSWER 3 OF 4 CAPLUS COPYRIGHT 2006 ACS on STN

TI Fluorescent halide indicators

L10 ANSWER 4 OF 4 CAPLUS COPYRIGHT 2006 ACS on STN

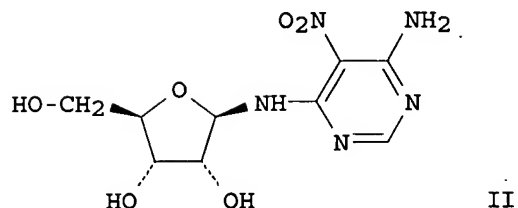
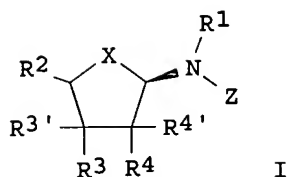
TI Long-wavelength iodide-sensitive fluorescent indicators for measurement of functional CFTR expression in cells

=> d l10 1 3 4 ti abs bib

L10 ANSWER 1 OF 4 CAPLUS COPYRIGHT 2006 ACS on STN

TI Preparation of nucleoside analogs for treating or preventing diseases associated with nonsense mutations of mRNA

GI



AB Nucleoside analogs I, wherein Z is (un)substituted alkyl, (un)substituted (un)substituted aryl, (un)substituted heteroaryl, (un)substituted cycloalkyl, (un)substituted heterocycle; X is CH, O, S, NH; R1 is H, (un)substituted alkyl, (un)substituted aryl, (un)substituted heteroaryl, (un)substituted cycloalkyl, (un)substituted heterocycle; R2 is (un)substituted alkyl, carboxy, amido, acyl, alkylcarbonyl, halogen, azide, alkyl amino, phosphate, phosphoester, alkyl ether; R3, R3', R4, R4' are independently (un)substituted ether, H, halogen, (un)substituted alkyl, (un)substituted (un)substituted aryl, (un)substituted heteroaryl, (un)substituted cycloalkyl, (un)substituted heterocycle are prepared for use in the treatment or prevention of diseases associated with nonsense mutations of mRNA. Thus, II was prepared and tested in a cell-based luciferase reporter assay containing a UGA premature termination codon that was stably transfected in 293T Human Embryonic Kidney cells (no data but very high potency and very high efficacy of protein synthesis). Further, I can be used as a prodrug in the treatment of autoimmune disease, blood diseases, collagen diseases, diabetes, neurodegenerative diseases, cardiovascular diseases, pulmonary diseases, inflammatory diseases, central nervous system diseases.

AN 2006:740594 CAPLUS

DN 145:167496

TI Preparation of nucleoside analogs for treating or preventing diseases associated with nonsense mutations of mRNA

IN Wilde, Richard G.; Almstead, Neil G.; Welch, Ellen M.; Beckmann, Holger

PA USA

SO U.S. Pat. Appl. Publ., 47 pp.

CODEN: USXXCO

DT Patent

LA English

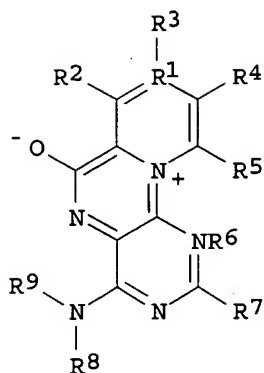
FAN.CNT 1

	PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI	US 2006166926	A1	20060727	US 2005-48659	20050121
PRAI	US 2005-48659		20050121		
OS	MARPAT 145:167496				

L10 ANSWER 3 OF 4 CAPLUS COPYRIGHT 2006 ACS on STN

TI Fluorescent halide indicators

GI



I

AB The invention provides methods and compns. for measuring ion concentration inside

a cell by measuring fluorescence of I (R1 = C, N'; R2-9 = (un)substituted alkyl, alkenyl, aryl, heteroalkyl, heteroaryl, acyl, etc.). In particular embodiments, the measured ion is halide, particularly iodide, the cell contains a functional anion transport protein or channel, the method measures a change in fluorescence as a function of a predetd. condition such as the presence of a predetd. amount of a candidate modulator of ion transport in the cell (e.g. for drug screening) or the expression by the cell of a transgene such as the cystic fibrosis CFTR gene (e.g. to assess the efficacy of gene therapy).

AN 2000:707146 CAPLUS

DN 133:278350

TI Fluorescent halide indicators

IN Verkman, Alan S.; Biwersi, Joachim; Jayaraman, Sujatha

PA The Regents of the University of California, USA

SO PCT Int. Appl., 50 pp.

CODEN: PIXXD2

DT Patent

LA English

FAN.CNT 1

	PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI	WO 2000058289	A1	20001005	WO 2000-US7799	20000324
	W: AE, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, CA, CH, CN, CR, CU, CZ, DE, DK, DM, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, NO, NZ, PL, PT, RO, RU, SD, SE, SG, SI, SK, SL, TJ, TM, TR, TT, TZ, UA, UG, UZ, VN, YU, ZA, ZW, AM, AZ, BY, KG, KZ, MD, RU, TJ, TM RW: GH, GM, KE, LS, MW, SD, SL, SZ, TZ, UG, ZW, AT, BE, CH, CY, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE, BF, BJ, CF, CG, CI, CM, GA, GN, GW, ML, MR, NE, SN, TD, TG				
	US 6201116	B1	20010313	US 1999-277354	19990326
PRAI	US 1999-277354	A	19990326		
OS	MARPAT 133:278350				

RE.CNT 3 THERE ARE 3 CITED REFERENCES AVAILABLE FOR THIS RECORD
ALL CITATIONS AVAILABLE IN THE RE FORMAT

L10 ANSWER 4 OF 4 CAPLUS COPYRIGHT 2006 ACS on STN

TI Long-wavelength iodide-sensitive fluorescent indicators for measurement of functional CFTR expression in cells

AB Limitations of available indicators [such as 6-methoxy-N-(3-sulfopropyl)quinolinium (SPQ)] for measurement of intracellular Cl⁻ are their relatively dim fluorescence and need for UV excitation. A series of long-wavelength polar fluorophores was screened to identify compds. with Cl⁻ and/or I⁻ sensitivity, bright fluorescence, low toxicity, uniform loading of cytoplasm with minimal leakage, and chemical stability in cells.

The best compound found was 7-(β -D-ribofuranosylamino)-pyrido[2,1-h]-pteridin-11-ium-5-olate (LZQ). LZQ is brightly fluorescent with excitation and emission maxima at 400-470 and 490-560 nm, molar extinction 11,100 M⁻¹·cm⁻¹ (424 nm), and quantum yield 0.53. LZQ fluorescence is quenched by I⁻ by a collisional mechanism (Stern-Volmer constant 60 M⁻¹) and is not affected by other halides, nitrate, cations, or pH changes (pH 5-8). After LZQ loading into cytoplasm by hypotonic shock or overnight incubation, LZQ remained trapped in cells (leakage <3%/h). LZQ stained cytoplasm uniformly, remained chemical inert, did not bind to cytoplasmic components, and was photobleached by <1% during 1 h of continuous illumination. Cytoplasmic LZQ fluorescence was quenched selectively by I⁻ (50% quenching at 38 mM I⁻). LZQ was used to measure forskolin-stimulated I⁻/Cl⁻ and I⁻/NO₃⁻ exchange in cystic fibrosis transmembrane conductance regulator (CFTR)-expressing cell lines by fluorescence microscopy and microplate reader instrumentation using 96-well plates. The substantially improved optical and cellular properties of LZQ over existing indicators should permit the quant. anal. of CFTR function in gene delivery trials and high-throughput screening of compds. for correction of the cystic fibrosis phenotype.

AN 1999:767665 CAPLUS

DN 132:90258

TI Long-wavelength iodide-sensitive fluorescent indicators for measurement of functional CFTR expression in cells

AU Jayaraman, Sujatha; Teitler, Leah; Skalski, Bohdan; Verkman, A. S.

CS Departments of Medicine and Physiology, Cardiovascular Research Institute, University of California, San Francisco, CA, 94143-0521, USA

SO American Journal of Physiology (1999), 277(5, Pt. 1), C1008-C1018

CODEN: AJPHAP; ISSN: 0002-9513

PB American Physiological Society

DT Journal

LA English

RE.CNT 37 THERE ARE 37 CITED REFERENCES AVAILABLE FOR THIS RECORD
ALL CITATIONS AVAILABLE IN THE RE FORMAT

=> d his

(FILE 'HOME' ENTERED AT 16:42:54 ON 23 OCT 2006)

FILE 'REGISTRY' ENTERED AT 16:43:05 ON 23 OCT 2006

L1 STRUCTURE UPLOADED

L2 18 S L1 SSS SAM

L3 2501 S L1 SSS FULL

FILE 'CAPLUS' ENTERED AT 16:44:30 ON 23 OCT 2006

L4 1317 S L3

L5 1 S L4 AND (NONSENSE(W) SUPPRES?)

FILE 'USPATFULL' ENTERED AT 16:45:11 ON 23 OCT 2006

L6 58 S L3

L7 0 S L6 AND (NONSNESE(W) SUPPRES?)

L8 0 S L6 AND (NONSENESE(W) SUPPRES?)

L9 2 S L6 AND (NONSENSE(W) SUPPRES?)

FILE 'CAPLUS' ENTERED AT 16:45:56 ON 23 OCT 2006

L10 4 S L4 AND ((MUSCULAR(W) DYSTROPHY) OR READTHROUGH OR (CYSTIC(W) FI

=> s l4 and (premature(w) stop)

30766 PREMATURE

39587 STOP

1762 PREMATURE(W) STOP

L11 0 L4 AND (PREMATURE(W) STOP)

=> s l4 and (stop(w) codon)

39587 STOP
37627 CODON
5402 STOP(W) CODON
L12 0 L4 AND (STOP(W) CODON)

=> index bioscience

FILE 'DRUGMONOG' ACCESS NOT AUTHORIZED
COST IN U.S. DOLLARS

	SINCE FILE ENTRY	TOTAL SESSION
FULL ESTIMATED COST	38.11	264.58

	SINCE FILE ENTRY	TOTAL SESSION
DISCOUNT AMOUNTS (FOR QUALIFYING ACCOUNTS)		
CA SUBSCRIBER PRICE	-2.25	-3.00

INDEX 'ADISCTI, ADISINSIGHT, ADISNEWS, AGRICOLA, ANABSTR, ANTE, AQUALINE,
AQUASCI, BIOENG, BIOSIS, BIOTECHABS, BIOTECHDS, BIOTECHNO, CABA, CAPLUS,
CEABA-VTB; CIN, CONFSCI, CROPB, CROPU, DDFB, DDFU, DGENE, DISSABS, DRUGB,
DRUGMONOG2, DRUGU, EMBAL, EMBASE, ...' ENTERED AT 16:50:08 ON 23 OCT 2006

68 FILES IN THE FILE LIST IN STNINDEX

Enter SET DETAIL ON to see search term postings or to view
search error messages that display as 0* with SET DETAIL OFF.

=> s (adenosine(w)kinase) and ((nonsense(w)suppres?) or readthrough)

24 FILES SEARCHED...

7 FILE GENBANK

1 FILE PROUSDDR

56 FILES SEARCHED...

2 FILES HAVE ONE OR MORE ANSWERS, 68 FILES SEARCHED IN STNINDEX

L13 QUE (ADENOSINE(W) KINASE) AND ((NONSENSE(W) SUPPRES?) OR READTHROUGH)

=> file prousddr

	SINCE FILE ENTRY	TOTAL SESSION
COST IN U.S. DOLLARS		
FULL ESTIMATED COST	1.22	265.80

	SINCE FILE ENTRY	TOTAL SESSION
DISCOUNT AMOUNTS (FOR QUALIFYING ACCOUNTS)		
CA SUBSCRIBER PRICE	0.00	-3.00

FILE 'PROUSDDR' ENTERED AT 16:51:19 ON 23 OCT 2006
COPYRIGHT (C) 2006 Prous Science

FILE COVERS 1980 TO 2 Oct 2006 (20061002/ED)

=> s (adenosine(w)kinase) and ((nonsense(w)suppres?) or readthrough)

1839 ADENOSINE

8611 KINASE

76 ADENOSINE(W) KINASE

10 NONSENSE

2806 SUPPRES?

6 NONSENSE(W) SUPPRES?

0 READTHROUGH

L14 1 (ADENOSINE(W) KINASE) AND ((NONSENSE(W) SUPPRES?) OR READTHROUGH)

=> d l14 1 ti abs bib

'ABS' IS NOT A VALID FORMAT FOR FILE 'PROUSDDR'

The following are valid formats:

The default display format is QRD.

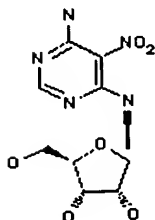
ALL ---- AN, DN, CN, RN, MF, PHP, STA, HDP(DSTA), CO, CC, ACTN,
OS, ED(UP), STR, PROUS REFERENCE: RE, RTX,
PATENT REFERENCES: TI, IN, PA, PI, PRAI, REFERENCE: RE
DALL --- Same as ALL with delimiters
IALL --- Indented ALL
BIB ---- AN, PROUS REFERENCE: RE, RTX,
PATENT REFERENCES: TI, IN, PA, PI, PRAI,
REFERENCE: RE
IBIB --- Indented BIB
IDE ---- AN, DN, CN, RN, MF, PHP, STA, HDP, CO, CC, OS, ED(UP), STR
IIDÉ --- Indented IDE
QRD ---- Query Related Data (AN, DN, CN, RN, MF, PHP, STA, HDP,
CO, CC, ACTN, OS, ED, STR, plus all fields containing hit terms)
SAM ---- Same as TRI
SCAN --- CC, CO, RN, CN, ACTN
TRIAL -- (TRI) AN, CN, CO, CC, ACTN, OS
FREE --- Same as TRI
HIT ---- All fields containing hit terms
KWIC --- All hit terms plus 20 words on either side
OCC ---- List of display fields containing hit terms

Hit terms will be highlighted in all displayable fields.

To display a particular field or fields, enter the display field codes. For a list of display fields codes, enter 'HELP DFIELDs' at an arrow prompt (=>). Examples of formats include: 'IALl';
ENTER DISPLAY FORMAT (QRD):qrd

L14 ANSWER 1 OF 1 PROUSDDR COPYRIGHT 2006 PROUS SCIENCE on STN
AN 1988:4248 PROUSDDR
DN 142010
CN 6-Amino-5-nitro-4-(beta-D-ribofuranosylamino)pyrimidine
CN GENERIC NAME: Clitocine
RN 105798-74-1
MF C9 H13 N5 O6
PHP M.p. 230-1 C
HDP BIOLOGICAL TESTING
CO ORIGINATOR: Valeant
CC Oncolytic Drugs
ACTN Antimetabolites
ED Entered STN: 9 May 2004
Last Updated on STN: 2 May 2006

STRUCTURE:



RTX RefID: 61515
ACTION - Antineoplastic agent, isolated from the mushroom Clitocybe inversa, which inhibits the growth of L1210 murine lymphocytic leukemia, WI-L2 human B-cell lymphoblastic leukemia and CCRF-CEM human TT-cell lymphoblastic leukemia in vitro (ID50 = 0.03 mcM), and also is a substrate and inhibitor of adenosine kinase (Ki = 3 mcM).

PATENT REFERENCES:

TI Use of nucleoside compounds for nonsense suppression
and the treatment of genetic diseases
IN Wilde, R.G.; Almstead, N.G.; Welch, E.M.; Beckmann, H.
PA Amgen
PA PTC Therapeutics
PI EP 1572709 20050914
WO 2004009610 20040129
PRAI US 2002-398334 20020724

START LOCAL KERMIT RECEIVE PROCESS

BINARY DATA HAS BEEN DOWNLOADED TO MULTIPLE FILES 'IMAGEennn.TIF'

=> log hold

COST IN U.S. DOLLARS

SINCE FILE	TOTAL
ENTRY	SESSION
23.41	289.21

FULL ESTIMATED COST

DISCOUNT AMOUNTS (FOR QUALIFYING ACCOUNTS)

SINCE FILE	TOTAL
ENTRY	SESSION
0.00	-3.00

CA SUBSCRIBER PRICE

SESSION WILL BE HELD FOR 120 MINUTES

STN INTERNATIONAL SESSION SUSPENDED AT 16:51:52 ON 23 OCT 2006

Connecting via Winsock to STN

Welcome to STN International! Enter x:x

LOGINID:SSPTAEXO1623

PASSWORD:

***** RECONNECTED TO STN INTERNATIONAL *****
SESSION RESUMED IN FILE 'PROUSDDR' AT 17:09:50 ON 23 OCT 2006
FILE 'PROUSDDR' ENTERED AT 17:09:50 ON 23 OCT 2006
COPYRIGHT (C) 2006 Prous Science

COST IN U.S. DOLLARS	SINCE FILE	TOTAL
	ENTRY	SESSION
FULL ESTIMATED COST	23.41	289.21
DISCOUNT AMOUNTS (FOR QUALIFYING ACCOUNTS)	SINCE FILE	TOTAL
	ENTRY	SESSION
CA SUBSCRIBER PRICE	0.00	-3.00

=> index bioscience

FILE 'DRUGMONOG' ACCESS NOT AUTHORIZED

COST IN U.S. DOLLARS	SINCE FILE	TOTAL
	ENTRY	SESSION
FULL ESTIMATED COST	23.41	289.21
DISCOUNT AMOUNTS (FOR QUALIFYING ACCOUNTS)	SINCE FILE	TOTAL
	ENTRY	SESSION
CA SUBSCRIBER PRICE	0.00	-3.00

INDEX 'ADISCTI, ADISINSIGHT, ADISNEWS, AGRICOLA, ANABSTR, ANTE, AQUALINE, AQUASCI, BIOENG, BIOSIS, BIOTECHABS, BIOTECHDS, BIOTECHNO, CABA, CAPLUS, CEABA-VTB, CIN, CONFSCI, CROPB, CROPU, DDFB, DDFU, DGENE, DISSABS, DRUGB, DRUGMONOG2, DRUGU, EMBAL, EMBASE, ...' ENTERED AT 17:10:06 ON 23 OCT 2006

68 FILES IN THE FILE LIST IN STNINDEX

Enter SET DETAIL ON to see search term postings or to view search error messages that display as 0* with SET DETAIL OFF.

=> s (gene(w)therapy) and (autoimmun? or arthrtis or (multiple(w)sclerosis) or (Alzheim?) or parkinson? or hemophilia or (ataxia-telangiectasia) or thalassemia or (kidney(w)stones))

6	FILE ADISCTI
52	FILE ADISINSIGHT
26	FILE ADISNEWS
7	FILE AGRICOLA
5	FILE ANTE
331	FILE BIOENG
1502	FILE BIOSIS
7290	FILE BIOTECHABS
7290	FILE BIOTECHDS
1426	FILE BIOTECHNO
13 FILES SEARCHED...	
19	FILE CABA
3295	FILE CAPLUS
64	FILE CEABA-VTB
195	FILE CIN
27	FILE CONFSCI
1	FILE CROPU
362	FILE DDFU

397280 FILE DGENE
 23 FILES SEARCHED...
 61 FILE DISSABS
 400 FILE DRUGU
 25 FILE EMBAL
 2232 FILE EMBASE
 938 FILE ESBIODBASE
 1 FILE FROSTI
 53 FILE GENBANK
 421 FILE IFIPAT
 186 FILE IMSDRUGNEWS

39 FILES SEARCHED...
 116 FILE IMSRESEARCH
 372 FILE JICST-EPLUS
 460 FILE LIFESCI
 1760 FILE MEDLINE
 15 FILE NTIS
 883 FILE PASCAL
 62 FILE PCTGEN
 43 FILE PHAR
 93 FILE PHARMAML
 1 FILE PHIC
 360 FILE PHIN
 2436 FILE PROMT
 4 FILE PROUSDDR

56 FILES SEARCHED...
 1 FILE RDISCLOSURE
 1951 FILE SCISEARCH
 864 FILE TOXCENTER
 14324 FILE USPATFULL
 1527 FILE USPAT2
 5447 FILE WPIDS
 32 FILE WPIFV
 5447 FILE WPINDEX

48 FILES HAVE ONE OR MORE ANSWERS, 68 FILES SEARCHED IN STNINDEX

L15 QUE (GENE(W) THERAPY) AND (AUTOIMMUN? OR ARTHRTIS OR (MULTIPLE(W) SCLEROSI
 S) OR (ALZHEIM?) OR PARKINSON? OR HEMOPHILIA OR (ATAXIA-TELANGIECTASIA
) OR THALASSEMIA OR (KIDNEY(W) STONES))

=> file embase medline caplus
 COST IN U.S. DOLLARS

SINCE FILE	TOTAL
ENTRY	SESSION
3.66	292.87

FULL ESTIMATED COST

DISCOUNT AMOUNTS (FOR QUALIFYING ACCOUNTS)

SINCE FILE	TOTAL
ENTRY	SESSION
0.00	-3.00

CA SUBSCRIBER PRICE

FILE 'EMBASE' ENTERED AT 17:13:44 ON 23 OCT 2006
 Copyright (c) 2006 Elsevier B.V. All rights reserved.

FILE 'MEDLINE' ENTERED AT 17:13:44 ON 23 OCT 2006

FILE 'CAPLUS' ENTERED AT 17:13:44 ON 23 OCT 2006
 USE IS SUBJECT TO THE TERMS OF YOUR STN CUSTOMER AGREEMENT.
 PLEASE SEE "HELP USAGETERMS" FOR DETAILS.
 COPYRIGHT (C) 2006 AMERICAN CHEMICAL SOCIETY (ACS)

=> s (gene(w)therapy) and (autoimmun? or arthrtis or (multiple(w)sclerosis) or
 (Alzheim?) or parkinson? or hemophilia or (ataxia-telangiectasia) or thalassemia or
 (kidney(w)stones))

L16 7287 (GENE(W) THERAPY) AND (AUTOIMMUN? OR ARTHRTIS OR (MULTIPLE(W)
 SCLEROSIS) OR (ALZHEIM?) OR PARKINSON? OR HEMOPHILIA OR (ATAXIA-

TELANGIECTASIA) OR THALASSEMIA OR (KIDNEY(W) STONES))

=> s l16 and clitocine

L17 0 L16 AND CLITOCINE

=> s l16 and (nonsense)

L18 14 L16 AND (NONSENSE)

=> d l18 1-14 ti

L18 ANSWER 1 OF 14 EMBASE COPYRIGHT (c) 2006 Elsevier B.V. All rights reserved on STN

TI Treatment of experimental autoimmune encephalomyelitis with antisense oligonucleotides against the low affinity neurotrophin receptor.

L18 ANSWER 2 OF 14 EMBASE COPYRIGHT (c) 2006 Elsevier B.V. All rights reserved on STN

TI Construction of a functional human suppressor tRNA gene: An approach to gene therapy for β -thalassaemia.

L18 ANSWER 3 OF 14 MEDLINE on STN

TI Novel hemophilia B mouse models exhibiting a range of mutations in the Factor IX gene.

L18 ANSWER 4 OF 14 MEDLINE on STN

TI Treatment of experimental autoimmune encephalomyelitis with antisense oligonucleotides against the low affinity neurotrophin receptor.

L18 ANSWER 5 OF 14 MEDLINE on STN

TI [Molecular genetics of hemophilia A].
Genetica molecular de la hemofilia A.

L18 ANSWER 6 OF 14 MEDLINE on STN

TI Construction of a functional human suppressor tRNA gene: an approach to gene therapy for beta-thalassaemia.

L18 ANSWER 7 OF 14 CAPLUS COPYRIGHT 2006 ACS on STN

TI RNA splicing manipulation: Strategies to modify gene expression for a variety of therapeutic outcomes

L18 ANSWER 8 OF 14 CAPLUS COPYRIGHT 2006 ACS on STN

TI Components of nonsense-mediated mRNA decay (NMD) complex, and methods of drug screening and treating disorders related to NMD by modulating NMD

L18 ANSWER 9 OF 14 CAPLUS COPYRIGHT 2006 ACS on STN

TI Treatment of experimental autoimmune encephalomyelitis with antisense oligonucleotides against the low affinity neurotrophin receptor

L18 ANSWER 10 OF 14 CAPLUS COPYRIGHT 2006 ACS on STN

TI A protein regulating decay of mRNAs containing nonsense codons and controlling the decay of foreign mRNAs in expression hosts using derivatives of the protein

L18 ANSWER 11 OF 14 CAPLUS COPYRIGHT 2006 ACS on STN

TI Positional cloning and sequence of the APECED gene associated with autoimmune polyendocrinopathy syndrome type I

L18 ANSWER 12 OF 14 CAPLUS COPYRIGHT 2006 ACS on STN

TI Molecular genetics of hemophilia A

L18 ANSWER 13 OF 14 CAPLUS COPYRIGHT 2006 ACS on STN

TI Gene therapy using homologous recombination for mutation correction

L18 ANSWER 14 OF 14 CAPLUS COPYRIGHT 2006 ACS on STN
TI Construction of a functional human suppressor tRNA gene: an approach to
gene therapy for β -thalassemia

=> d l18 1-18 ti abs bib

L18 ANSWER 1 OF 14 EMBASE COPYRIGHT (c) 2006 Elsevier B.V. All rights
reserved on STN
TI Treatment of experimental autoimmune encephalomyelitis with
antisense oligonucleotides against the low affinity neurotrophin receptor.
AB Upregulated expression of the low-affinity neurotrophin receptor (p75) in
the central nervous system (CNS) during experimental autoimmune
encephalomyelitis (EAE) has recently been demonstrated. To investigate
whether p75 plays a role in disease pathogenesis, we adopted a
gene therapy approach, utilizing antisense
oligonucleotides to downregulate p75 expression during EAE.
Phosphorothioate antisense oligonucleotides (AS), nonsense
oligonucleotides (NS) or phosphate buffered saline (PBS) were injected
daily for 18 days after immunization of SJL/J (H-2s)-mice with myelin
proteolipid protein (PLP) peptide 139-151. In the AS group, there was a
statistically significant reduction in both the mean maximal disease score
(1.85 in the AS, 2.94 in the NS and 2.75 in the PBS-groups, respectively,
 $P < 0.025$) and in the cumulative disease incidence (.simeq. 60% in the AS
group and .simeq. 90% in the control groups). Histological and
immunohistochemical analysis showed reduced inflammation and
demyelination, as well as reduced p75 expression at the blood-brain
barrier (BBB) in the AS-treated mice in comparison with both control
groups. There was no difference, however, in p75 expression on neural
cells within the CNS between the three groups of mice. We conclude that
p75 could play a proactive role in the pathogenesis of EAE and may exert
its effect at the level of the BBB. (C) 2000 Wiley-Liss, Inc.
AN 2000358373 EMBASE
TI Treatment of experimental autoimmune encephalomyelitis with
antisense oligonucleotides against the low affinity neurotrophin receptor.
AU Soilu-Hanninen M.; Epa R.; Shipham K.; Butzkueven H.; Bucci T.; Barrett
G.; Bartlett P.F.; Kilpatrick T.J.
CS Dr. T.J. Kilpatrick, Development and Neurobiology Group, Walter/Eliza Hall
Inst. of Med. Res., Royal Melbourne Hospital, Parkville, Vic. 3050,
Australia. kilpatrick@wehi.edu.au
SO Journal of Neuroscience Research, (15 Mar 2000) Vol. 59, No. 6, pp.
712-721. .
Refs: 41
ISSN: 0360-4012 CODEN: JNREDK
CY United States
DT Journal; Article
FS 005 General Pathology and Pathological Anatomy
008 Neurology and Neurosurgery
026 Immunology, Serology and Transplantation
LA English
SL English
ED Entered STN: 2 Nov 2000
Last Updated on STN: 2 Nov 2000

L18 ANSWER 2 OF 14 EMBASE COPYRIGHT (c) 2006 Elsevier B.V. All rights
reserved on STN
TI Construction of a functional human suppressor tRNA gene: An approach to
gene therapy for β -thalassaemia.
AB A human rRNA(Lys) gene was converted to an amber suppressor by
site-specific mutagenesis of the anticodon. The mutated tRNA(Lys) gene
directed synthesis of a tRNA suppressed the UAG amber nonsense
mutation in β 0 thalassaemia mRNA. Such genes may be used to detect
other nonsense mutations in mammalian cells and may provide an
approach to gene therapy for β 0 thalassaemia due
to nonsense mutations.

AN 82206283 EMBASE
 DN 1982206283
 TI Construction of a functional human suppressor tRNA gene: An approach to gene therapy for β -thalassaemia.
 AU Temple G.F.; Dozy A.M.; Roy K.L.; Kan Y.W.
 CS Howard Hughes Med. Inst. Lab., Univ. California, San Francisco, CA 94143, United States
 SO Nature, (1982) Vol. 296, No. 5857, pp. 537-540. .
 CODEN: NATUAS
 CY United Kingdom
 DT Journal
 FS 022 Human Genetics
 025 Hematology
 LA English
 ED Entered STN: 9 Dec 1991
 Last Updated on STN: 9 Dec 1991

L18 ANSWER 3 OF 14 MEDLINE on STN
 TI Novel hemophilia B mouse models exhibiting a range of mutations in the Factor IX gene.
 AB Animal models have been critical to the development of novel therapeutics in hemophilia. A deficiency of current murine models of hemophilia B is that they are all due to gene deletions, a type of mutation that is relatively rare in the human hemophilia population. We generated mice with a range of mutations in the Factor IX (F.IX) gene; these more faithfully reflect the types of mutations that cause disease in the human population. Transgenic mice expressing either wild-type human F.IX (hF.IX), or F.IX variants with premature translation termination codons, or missense mutations, under the control of the murine transthyretin promoter, were generated and crossed with mice carrying a large deletion of the murine F.IX gene. Gene copy number, F.IX transcript levels in the liver, intrahepatocyte protein expression, and circulating levels of F.IX protein in the mice were determined and compared with data generated by transient transfection assays using the same F.IX variants. Mice were injected with a viral vector expressing hF.IX and displayed a range of immune responses to the transgene product, depending on the underlying mutation. These new mouse models faithfully mimic the mutations causing human disease, and will prove useful for testing novel therapies for hemophilia.

AN 2004527510 MEDLINE
 DN PubMed ID: 15217833
 TI Novel hemophilia B mouse models exhibiting a range of mutations in the Factor IX gene.
 AU Sabatino Denise E; Armstrong Elina; Edmonson Shyrie; Liu Yi-Lin; Pleimes Marc; Schuettrumpf Joerg; Fitzgerald Julie; Herzog Roland W; Arruda Valder R; High Katherine A
 CS Department of Pediatrics, Graduate Program in Gene Therapy, University of Pennsylvania School of Medicine, Philadelphia, USA.
 NC K01 DK60580 (NIDDK)
 P01 HL64190 (NHLBI)
 R01A1/HL51390 (NHLBI)
 U01 HL66948 (NHLBI)
 SO Blood, (2004 Nov 1) Vol. 104, No. 9, pp. 2767-74. Electronic Publication: 2004-06-24.
 Journal code: 7603509. ISSN: 0006-4971.
 CY United States
 DT Journal; Article; (JOURNAL ARTICLE)
 LA English
 FS Abridged Index Medicus Journals; Priority Journals
 EM 200412
 ED Entered STN: 23 Oct 2004
 Last Updated on STN: 20 Dec 2004
 Entered Medline: 14 Dec 2004

L18 ANSWER 4 OF 14 MEDLINE on STN

TI Treatment of experimental autoimmune encephalomyelitis with
antisense oligonucleotides against the low affinity neurotrophin receptor.
AB Upregulated expression of the low-affinity neurotrophin receptor (p75) in
the central nervous system (CNS) during experimental autoimmune
encephalomyelitis (EAE) has recently been demonstrated. To investigate
whether p75 plays a role in disease pathogenesis, we adopted a
gene therapy approach, utilizing antisense
oligonucleotides to downregulate p75 expression during EAE.
Phosphorothioate antisense oligonucleotides (AS), nonsense
oligonucleotides (NS) or phosphate buffered saline (PBS) were injected
daily for 18 days after immunization of SJL/J (H-2s)-mice with myelin
proteolipid protein (PLP) peptide 139-151. In the AS group, there was a
statistically significant reduction in both the mean maximal disease score
(1.85 in the AS, 2.94 in the NS and 2.75 in the PBS-groups, respectively,
P < 0.025) and in the cumulative disease incidence (approximately 60% in
the AS group and approximately 90% in the control groups). Histological
and immunohistochemical analysis showed reduced inflammation and
demyelination, as well as reduced p75 expression at the blood-brain
barrier (BBB) in the AS-treated mice in comparison with both control
groups. There was no difference, however, in p75 expression on neural
cells within the CNS between the three groups of mice. We conclude that
p75 could play a proactive role in the pathogenesis of EAE and may exert
its effect at the level of the BBB.

Copyright 2000 Wiley-Liss, Inc.

AN 2000164869 MEDLINE

DN PubMed ID: 10700008

TI Treatment of experimental autoimmune encephalomyelitis with
antisense oligonucleotides against the low affinity neurotrophin receptor.

AU Soilu-Hanninen M; Epa R; Shipham K; Butzkueven H; Bucci T; Barrett G;
Bartlett P F; Kilpatrick T J

CS The Walter and Eliza Hall Institute of Medical Research, The Royal
Melbourne Hospital, Parkville, Victoria, Australia.

SO Journal of neuroscience research, (2000 Mar 15) Vol. 59, No. 6, pp.
712-21.

Journal code: 7600111. ISSN: 0360-4012.

CY United States

DT Journal; Article; (JOURNAL ARTICLE)

LA English

FS Priority Journals

EM 200004

ED Entered STN: 21 Apr 2000

Last Updated on STN: 21 Apr 2000

Entered Medline: 13 Apr 2000

L18 ANSWER 5 OF 14 MEDLINE on STN

TI [Molecular genetics of hemophilia A].

Genetica molecular de la hemofilia A.

AB Hemophilia A (HemA), an X linked genetic disease, is the most
common coagulation disorder with an incidence of about 1-2 in 10,000 males
and is caused by mutations in the factor VIII (FVIII) coagulation gene.
Firstly, some clinical aspects of the HemA are presented: the current
methods to assess both the amount and activity of FVIII, the severity
range observed and the presence of inhibitor antibodies against the
therapeutic FVIII. Follows a discussion of the relationship of the
structural domains of the FVIII protein (Figure 1), the aminoacid sequence
and their functions. An activation-inactivation model of the successive
peptide bonds cleavages of the FVIII is also presented (Figure 2). After
the cloning of the FVIII gene in 1984, almost all types of HemA causing
mutations have been characterized. However, the size and complexity of
this gene prevented a screening of the full range of mutations for an
accurate molecular diagnosis. Moreover, most of the patients with
moderate and mild disease have missense mutations whereas approximately
half of severe patients have nonsense, frameshift, and some
missense mutations. There are also less frequently mutations such as
deletions and insertions leading to severe phenotype and mutations

affecting mRNA splicing and duplications causing both severe and mild HemA. In order to give genetic counselling in HemA families, studies at the DNA level using intragenic and/ or extragenic polymorphism analysis have been used. But this approach is not entirely satisfactory because it fails in several situations. Most of the causing mutations described above are private, and they have been found in only a few unrelated families. Recently, a common molecular inversion of the FVIII gene was identified in 50% of unrelated patients with severe HemA. The copies of a particular DNA sequence (termed F8A gene). One copy is located within intron 22 of the FVIII gene and the other two, 500 kb upstream. An homologous recombination mechanism was proposed for the inversion between an intragenic copy of the F8A gene and either the distal (80% of the inversion) or the proximal copy (20%). Both of these inversions lead to severe HemA because no intact FVIII is produced and can be easily diagnosed by Southern blot analysis. This inversion originates almost exclusively in male germ cells, because pairing Xq with its homologous in female meiosis would probably inhibit the proposed intrachromosome recombination. The molecular analysis of the inversion of intron 22 is now considered as the first line for families with severe HemA patients. In recent years the treatment of patients with hemophilia A and B has been intravenous injection of FVIII or FIX concentrates, respectively. This regimen of regular injection of plasmatic proteins bears a high risk of infection by contaminating viruses (HIV, HBV, etc). Future treatment for patients with hemophilia may include the use of either gene therapy or recombinant coagulation factors. Both strategies would completely avoid the infection risk offering a safe and effective treatment for the disease. Recombinant factors, obtained by genetic engineering methods, provide a renewable and unlimited source of FVIII or FIX. The clinical trials of recombinant factors have already started in mid-1995 giving positive results. On the other hand, gene therapy for hemophilia is now in the pre-clinical stage but offers the prospect of a cure for the disease, thus potentially freeing patients from regular injections of the lacking protein. However, experiments in animal models suggest that it may be difficult to obtain adequate therapeutic levels of factors for long periods of time. Recently, a retroviral-mediated gene delivery of human FVIII in mice has been reported using the ex vivo strategy of gene therapy. Therapeutic levels of FVIII in the circulation were obtained for > 1 week and it was also observed that the capacity of primary cells to deliver FVIII in blood was strongly dependent on

AN 97384041 MEDLINE
 DN PubMed ID: 9239887
 TI [Molecular genetics of hemophilia A].
 TI Genetica molecular de la hemofilia A.
 AU De Brasi C D; Slavutsky I R; Larripa I B
 CS Departamento de Genetica, Academia Nacional de Medicina, Buenos Aires,
 Argentina.
 SO Medicina, (1996) Vol. 56, No. 5 Pt 1, pp. 509-17. Ref: 32
 Journal code: 0204271. ISSN: 0025-7680.
 CY Argentina
 DT Journal; Article; (JOURNAL ARTICLE)
 DT General Review; (REVIEW)
 LA Spanish
 FS Priority Journals
 EM 199710
 ED Entered STN: 24 Dec 1997
 Last Updated on STN: 29 Jan 1999
 Entered Medline: 27 Oct 1997

L18 ANSWER 6 OF 14 MEDLINE on STN
 TI Construction of a functional human suppressor tRNA gene: an approach to
 gene therapy for beta-thalassaemia.
 AB A human tRNA^{Lys} gene was converted to an amber suppressor by site-specific
 mutagenesis of the anticodon. The mutated tRNA^{Lys} gene directed synthesis
 of a tRNA that suppressed the UAG amber nonsense mutation in

beta O thalassemia mRNA. Such genes may be used to detect other nonsense mutations in mammalian cells and may provide an approach to gene therapy for beta O thalassaemia due to nonsense mutations.

AN 82173152 MEDLINE
DN PubMed ID: 6803169
TI Construction of a functional human suppressor tRNA gene: an approach to gene therapy for beta-thalassaemia.
AU Temple G F; Dozy A M; Roy K L; Kan Y W
SO Nature, (1982 Apr 8) Vol. 296, No. 5857, pp. 537-40.
Journal code: 0410462. ISSN: 0028-0836.
CY ENGLAND: United Kingdom
DT Journal; Article; (JOURNAL ARTICLE)
LA English
FS Priority Journals
EM 198206
ED Entered STN: 17 Mar 1990
Last Updated on STN: 17 Mar 1990
Entered Medline: 21 Jun 1982

L18 ANSWER 7 OF 14 CAPLUS COPYRIGHT 2006 ACS on STN

TI RNA splicing manipulation: Strategies to modify gene expression for a variety of therapeutic outcomes
AB A review. Antisense oligonucleotides initially offered great hope as specific compds. to modify gene expression, primarily through RNaseH induced degradation of the target transcript. Expansion of the field led to new chemistries capable of invoking different mechanisms, including suppression of protein synthesis by translational blockade, and there is now a major interest in downregulation of gene expression using short interfering RNAs to induce RNA silencing. Naturally occurring microRNAs have been implicated in the regulation of gene expression. This review considers examples of antisense oligonucleotides redirecting the process of exon recognition and intron removal during gene transcript splicing. While suppression of gene expression is necessary to address some conditions, it appears likely that there may be many more clin. applications for antisense oligonucleotides in re-directing splicing patterns. Pre-mRNA splicing is a tightly coordinated, multifactorial process, which can be disrupted by antisense oligonucleotides in a highly specific manner, allowing either suppression of aberrant splicing, bypass of nonsense or frame-shifting mutations or alteration of spliceoform ratios. Manipulation of splicing patterns has been applied to a diverse range of conditions, including β -thalassemia, Duchenne muscular dystrophy, spinal muscular atrophy and certain cancers. Alternative exon usage has been identified as a major mechanism for generating diversity from a limited repertoire of genes in higher eukaryotes. Considering that up to 75% of all human primary gene transcripts are reported to be alternatively spliced, intervention at the level of pre-mRNA processing is likely to become increasingly significant in the fight against genetic and acquired disorders.

AN 2005:1093515 CAPLUS
DN 144:247820
TI RNA splicing manipulation: Strategies to modify gene expression for a variety of therapeutic outcomes
AU Wilton, Steve D.; Fletcher, Susan
CS Experimental Molecular Medicine Group, Centre for Neuromuscular and Neurological Disorders, University of Western Australia, Australia
SO Current Gene Therapy (2005), 5(5), 467-483
CODEN: CGTUAH; ISSN: 1566-5232
PB Bentham Science Publishers Ltd.
DT Journal; General Review
LA English

RE.CNT 161 THERE ARE 161 CITED REFERENCES AVAILABLE FOR THIS RECORD
ALL CITATIONS AVAILABLE IN THE RE FORMAT

L18 ANSWER 8 OF 14 CAPLUS COPYRIGHT 2006 ACS on STN

TI Components of nonsense-mediated mRNA decay (NMD) complex, and methods of drug screening and treating disorders related to NMD by modulating NMD

AB Disclosed are compns. and methods relating to nonsense mediated decay (NMD) particularly in the 'pioneer' round of translation. Disclosed are methods of screening for a substance that modulates a NMD complex. The complex optionally comprises Upf2, Upf3, Upf3X, PABP2, CBP20, CBP80, hSmg5/7a, hSmg5/7b, hSmg5/7c, poly(A)RNase (PARN), Dcp2, PM/Sc1100, Rat1, Xrn1, Rrp41 or a combination thereof. The present invention provides methods of treating disorders relating to NMD and methods of screening substances for use in the study of or treatment of conditions relating to NMD.

AN 2004:371048 CAPLUS

DN 140:368631

TI Components of nonsense-mediated mRNA decay (NMD) complex, and methods of drug screening and treating disorders related to NMD by modulating NMD

IN Maquat, Lynne E.

PA University of Rochester, USA

SO PCT Int. Appl., 192 pp.

CODEN: PIXXD2

DT Patent

LA English

FAN.CNT 1

	PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI	WO 2004037976	A2	20040506	WO 2003-US26166	20030821
	WO 2004037976	A3	20040805		
	W:				
	AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW				
	RW:				
	GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW, AM, AZ, BY, KG, KZ, MD, RU, TJ, TM, AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR, BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG				
	AU 2003298555	A1	20040513	AU 2003-298555	20030821
	US 2006154883	A1	20060713	US 2006-525273	20060207
PRAI	US 2002-405602P	P	20020822		
	WO 2003-US26166	W	20030821		

L18 ANSWER 9 OF 14 CAPLUS . COPYRIGHT 2006 ACS on STN

TI Treatment of experimental autoimmune encephalomyelitis with antisense oligonucleotides against the low affinity neurotrophin receptor

AB Upregulated expression of the low-affinity neurotrophin receptor (p75) in the central nervous system (CNS) during exptl. autoimmune encephalomyelitis (EAE) has recently been demonstrated. To investigate whether p75 plays a role in disease pathogenesis, the authors adopted a gene therapy approach, utilizing antisense oligonucleotides to downregulate p75 expression during EAE. Phosphorothioate antisense oligonucleotides (AS), nonsense oligonucleotides (NS) or phosphate buffered saline (PBS) were injected daily for 18 days after immunization of SJL/J (H-2s)-mice with myelin proteolipid protein (PLP) peptide 139-151. In the AS group, there was a statistically significant reduction in both the mean maximal disease score (1.85 in the AS, 2.94 in the NS and 2.75 in the PBS-groups, resp.) and in the cumulative disease incidence ($\approx 60\%$ in the AS group and $\approx 90\%$ in the control groups). Histol. and immunohistochem. anal. showed reduced inflammation and demyelination, as well as reduced p75 expression at the blood-brain barrier (BBB) in the AS-treated mice in comparison with both control groups. There was no difference, however, in p75 expression on neural cells within the CNS between the three groups of mice. The authors conclude that p75 could play a proactive role in the

pathogenesis of EAE and may exert its effect at the level of the BBB.
 AN 2000:182651 CAPLUS
 DN 132:342990
 TI Treatment of experimental autoimmune encephalomyelitis with
 antisense oligonucleotides against the low affinity neurotrophin receptor
 AU Soilu-Hanninen, Merja; Epa, Ruwan; Shiphram, Kylie; Butzkueven, Helmut;
 Bucci, Tamara; Barrett, Graham; Bartlett, Perry F.; Kilpatrick, Trevor J.
 CS The Walter and Eliza Hall Institute of Medical Research, The Royal
 Melbourne Hospital, Parkville, 3050, Australia
 SO Journal of Neuroscience Research (2000), 59(6), 712-721
 CODEN: JNREDK; ISSN: 0360-4012
 PB Wiley-Liss, Inc.
 DT Journal
 LA English
 RE.CNT 41 THERE ARE 41 CITED REFERENCES AVAILABLE FOR THIS RECORD
 ALL CITATIONS AVAILABLE IN THE RE FORMAT

L18 ANSWER 10 OF 14 CAPLUS COPYRIGHT 2006 ACS on STN
 TI A protein regulating decay of mRNAs containing nonsense codons
 and controlling the decay of foreign mRNAs in expression hosts using
 derivatives of the protein
 AB A Saccharomyces cerevisiae gene, NMD2, named after its role in the
 nonsense-mediated mRNA decay pathway, and the gene product are
 characterized. The NMD2 gene product binds to another protein involved in
 this decay pathway, the UPF1 gene product. A C-terminal fragment of the
 NMD2 protein is shown to bind the UPF1 protein and when a gene for this
 fragment is overexpressed in the host cell, the fragment inhibits the
 function of UPF1 and blocks the nonsense-mediated mRNA decay
 pathway. This may be used to block the degradation of mRNAs containing a
 nonsense codon that would normally cause an increase in the
 transcript decay rate. Such stabilization of a transcript is useful in
 manufacture the manufacture of proteins encoded by genes containing an
 unexpected
 nonsense codon, e.g. in the manufacture of a fragment of a protein
 generated by introduction of a nonsense mutation into a gene.
 The invention also relates to methods of identifying mols. that inhibit
 the nonsense-mediated mRNA decay pathway, and the use of such
 mols. for treatment of disorders associated with nonsense
 mutations.

AN 1999:286119 CAPLUS
 DN 130:308197
 TI A protein regulating decay of mRNAs containing nonsense codons
 and controlling the decay of foreign mRNAs in expression hosts using
 derivatives of the protein
 IN He, Feng; Jacobson, Allan S.
 PA University of Massachusetts, USA
 SO PCT Int. Appl., 117 pp.
 CODEN: PIXXD2
 DT Patent
 LA English

FAN.CNT 3

	PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI	WO 9920797	A1	19990429	WO 1998-US22365	19981021
	W: AU, CA, JP, KR				
	RW: AT, BE, CH, CY, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE				
	AU 9911140	A1	19990510	AU 1999-11140	19981021
PRAI	US 1997-955472	A	19971021		
	WO 1998-US22365	W	19981021		

RE.CNT 1 THERE ARE 1 CITED REFERENCES AVAILABLE FOR THIS RECORD
 ALL CITATIONS AVAILABLE IN THE RE FORMAT

L18 ANSWER 11 OF 14 CAPLUS COPYRIGHT 2006 ACS on STN
 TI Positional cloning and sequence of the APECED gene associated with

autoimmune polyendocrinopathy syndrome type I

AB The present invention relates to a novel gene, a novel protein encoded by said gene, a mutated form of the gene and to diagnostic and therapeutic uses of the gene or a mutated form thereof. More specifically, the present invention relates to a novel gene defective in autoimmune polyendocrinopathy syndrome type I (APS I), also called autoimmune polyendocrinopathy-candidiasis-ectodermal dystrophy (APECED) (MIM Number 240,300). Autoimmune polyglandular syndrome type I (APS I, also called APECED) is an autosomal-recessive disorder that maps to human chromosome 21q22.3 between markers D21S49 and D21S171 by linkage studies. A novel gene was isolated from this region, AIRE (autoimmune regulator), which encodes a protein containing motifs suggestive of a transcription factor including two zinc-finger (PHD-finger) motifs, a proline-rich region and three LXXLL motifs. Two mutations, a C→T substitution that changes the Arg 257 (CGA) to a stop codon (TGA) and an A→G substitution that changes the Lys 83 (AAG) to a Glu codon (GAG), were found in this novel gene in Swiss and Finnish APECED patients. The Arg257stop (R257X) is the predominant mutation in Finnish APECED patients, accounting for 10/12 alleles studied. These results indicate that this gene is responsible for the pathogenesis of APECED. The identification of the gene defective in APECED should facilitate the genetic diagnosis and potential treatment of the disease and further enhance our general understanding of the mechanisms underlying autoimmune diseases.

AN 1999:222961 CAPLUS

DN 130:247887

TI Positional cloning and sequence of the APECED gene associated with autoimmune polyendocrinopathy syndrome type I

IN Krohn, Kai; Heino, Maarit; Peterson, Part; Scott, Hamish; Antonarakis, Stylianos; Lalioti, Maria; Shimizu, Nobuyoshi; Kudoh, Jun

PA Finnish Immunotechnology Ltd., Finland

SO PCT Int. Appl., 59 pp.

CODEN: PIXXD2

DT Patent

LA English

FAN.CNT 1

	PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI	WO 9915559	A1	19990401	WO 1998-FI749	19980923
	W:	AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, CA, CH, CN, CU, CZ, DE, DK, EE, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MD, MG, MK, MN, MW, MX, NO, NZ, PL, PT, RO, RU, SD, SE, SG, SI, SK, SL, TJ, TM, TR, TT, UA, UG, US, UZ, VN, YU, ZW, AM, AZ, BY, KG, KZ, MD, RU, TJ, TM			
	RW:	GH, GM, KE, LS, MW, SD, SZ, UG, ZW, AT, BE, CH, CY, DE, DK, ES, FI, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE, BF, BJ, CF, CG, CI, CM, GA, GN, GW, ML, MR, NE, SN, TD, TG			
	AU 9893502	A1	19990412	AU 1998-93502	19980923
	EP 1017718	A1	20000712	EP 1998-946476	19980923
	R:	AT, BE, CH, DE, DK, ES, FR, GB, GR, IT, LI, LU, NL, SE, MC, PT, IE, FI			

PRAI FI 1997-3762 A 19970923

WO 1998-FI749 W 19980923

RE.CNT 3 THERE ARE 3 CITED REFERENCES AVAILABLE FOR THIS RECORD
ALL CITATIONS AVAILABLE IN THE RE FORMAT

L18 ANSWER 12 OF 14 CAPLUS COPYRIGHT 2006 ACS on STN

TI Molecular genetics of hemophilia A

AB A review with 32 refs. Hemophilia A (HemA), an X-linked genetic disease, is the most common coagulation disorder with an incidence of about 1-2 in 10,000 males and is caused by mutations in the factor VIII (FVIII) coagulation gene. Firstly, some clin. aspects of the HemA are presented: the current methods to assess both the amount and activity of FVIII, the severity range observed, and the presence of inhibitor antibodies against the therapeutic FVIII. A discussion of the relation of the

structural domains of the FVIII protein and of the amino acid sequence and their functions follows. An activation-inactivation model of the successive peptide bonds cleavages of the FVIII is also presented. After the cloning of the FVIII gene in 1984, almost all types of HemA causing mutations have been characterized. However, the size and complexity of this gene prevented a screening of the full range of mutations for an accurate mol. diagnosis. Moreover, most of the patients with moderate and mild disease have missense mutations, whereas approx. half of severe patients have nonsense, frameshift, and some missense mutations. There are also less frequently mutations such as deletions and insertions leading to severe phenotype and mutations affecting mRNA splicing and duplications causing both severe and mild HemA. In genetic counseling of HemA families, studies at the DNA level using intragenic and/or extragenic polymorphism anal. have been used. But this approach is not entirely satisfactory because it fails in several situations. Most of the causing mutations described above are private, and they have been found in only a few unrelated families. Recently, a common mol. inversion of the FVIII gene was identified in 50% of unrelated patients with severe HemA. The inversion is mediated by the presence of three copies of a particular DNA sequence (termed F8A gene). One copy is located within intron 22 of the FVIII gene and the other two, 500 kb upstream. An homologous recombination mechanism was proposed for the inversion between an intragenic copy of the F8A gene and either the distal (80% of the inversion) or the proximal copy (20%). Both of these inversions lead to severe HemA because no intact FVIII is produced and can be easily diagnosed by Southern blot anal. This inversion originates almost exclusively in male germ cells, because pairing Xq with its homolog in female meiosis would probably inhibit the proposed intrachromosome recombination. The mol. anal. of the inversion of intron 22 is now considered as the first line for families with severe HemA patients. In recent years, the treatment of patients with hemophilia A and B has been i.v. injection of FVIII or FIX concs., resp. This regimen of regular injection of plasmatic proteins bears a high risk of infection by contaminating viruses (HIV, HBV, etc.). Future treatment for patients with hemophilia may include the use of either gene therapy or recombinant coagulation factors. Both strategies would completely avoid the infection risk offering a safe and effective treatment for the disease. Recombinant factors, obtained by genetic engineering methods, provide a renewable and unlimited source of FVIII or FIX. The clin. trials of recombinant factors have already started in mid-1995 giving pos. results. Gene therapy for hemophilia is now in the pre-clin. stage but offers the prospect of a cure for the disease, thus potentially freeing patients from regular injections of the lacking protein. However, expts. in animal models suggest that it may be difficult to obtain adequate therapeutic levels of factors for long periods of time. Recently, a retroviral-mediated gene delivery of human FVIII in mice has been reported using the ex vivo strategy of gene therapy. Therapeutic levels of FVIII in the circulation were obtained for >1 wk and it was also observed that the capacity of primary cells to deliver FVIII in blood was strongly dependent on the site of implantation. Although much work remains to be done, these pos. results are encouraging for the future of gene therapy for this relatively common genetic disease.

AN 1997:137032 CAPLUS

DN 126:184435

TI Molecular genetics of hemophilia A

AU De Brasi, Carlos D.; Slavutsky, Irma R.; Larripa, Irene B.

CS Dep. Genet., Acad. Nacional Med., Buenos Aires, Argent.

SO Medicina (Buenos Aires) (1996), 56(5/1), 509-517

CODEN: MEDCAD; ISSN: 0025-7680

PB Sociedad Argentina de Investigacion Clinica

DT Journal; General Review

LA Spanish

TI Gene therapy using homologous recombination for mutation correction

AB A method to correct is provided to correct the mutation(s) behind a genetic disease in the genome of somatic cells derived from an individual afflicted with this disease by contacting the cells with the corresponding non-mutant DNA-fragment to allow it to undergo homologous recombination and, thus, replace a DNA sequence of the somatic cell genome, wherein the mutation(s) is(are) located. The cells obtained can be administered to an individual as a treatment of the disease. A DNA-liposome suspension comprising the non-mutant DNA-fragment can be used as a DNA-vehicle in the process. In addition, it can be administered to an individual to obtain correction of mutation(s) by in vivo integration of the said DNA into a mutated gene by homologous recombination. Diseases for which the responsible mutations have been identified and which, thus, could be treated by the above method are autosomal and X-linked genetic disorders, such as von Willebrand's disease, sickle-cell anemia, β -thalassemia, hemophilia A and B, and cystic fibrosis. Thus, lymphocytes from a patient with a homozygous nonsense mutation (R1659X/R1659X) in exon 28 of the von Willebrand factor gene were used to establish Epstein-Barr virus-transformed lymphocytes. A non-mutant DNA fragment comprised of the whole exon 28 and parts of introns 27 and 28 and corresponding to the mutant region, was amplified from normal individuals, cloned, and used to feed a culture of the above lymphocytes. Transfer of the non-mutant DNA fragment into the lymphocytes was mediated by lipofectamine, a pos. charged liposome. After the cells were fed 14 times, the repaired cells amounted roughly to 0.5% of the total tested cells.

AN 1996:295077 CAPLUS

DN 124:309568

TI Gene therapy using homologous recombination for mutation correction

IN Anvret, Maria; Blombaeck, Margareta; Zhang, Zhiping

PA Swed.

SO PCT Int. Appl., 19 pp.

CODEN: PIXXD2

DT Patent

LA English

FAN.CNT 1

	PATENT NO.	KIND	DATE	APPLICATION NO.	DATE
PI	WO 9604397	A1	19960215	WO 1994-SE1038	19941103
	W: AM, AT, AU, BB, BG, BR, BY, CA, CH, CN, CZ, DE, DK, EE, ES, FI, GB, GE, HU, JP, KE, KG, KP, KR, KZ, LK, LT, LU, LV, MD, MG, MN, MW, NL, NO, NZ, PL, PT, RO, RU, SD, SE, SI, SK, TJ, TT, UA, UZ, VN, RW: KE, MW, SD, SZ, AT, BE, CH, DE, DK, ES, FR, GB, GR, IE, IT, LU, MC, NL, PT, SE, BF, BJ, CF, CG, CI, CM, GA, GN, ML, MR, NE, SN, TD, TG				
	AU 9512854	A1	19960304	AU 1995-12854	19941103
PRAI	SE 1994-2642	A	19940805		
	WO 1994-SE1038	W	19941103		

L18 ANSWER 14 OF 14 CAPLUS COPYRIGHT 2006 ACS on STN

TI Construction of a functional human suppressor tRNA gene: an approach to gene therapy for β -thalassemia

AB A human tRNA^{Lys} gene was converted to an amber suppressor by site-specific mutagenesis of the anticodon. The mutated tRNA^{Lys} gene directed synthesis of a tRNA that suppressed the UAG amber nonsense mutation in β^0 -thalassemia mRNA. Such genes may be used to detect other nonsense mutations in mammalian cells and may provide an approach to gene therapy for β^0 -thalassemia due to nonsense mutations.

AN 1982:450555 CAPLUS

DN 97:50555

TI Construction of a functional human suppressor tRNA gene: an approach to gene therapy for β -thalassemia

AU Temple, Gary F.; Dozy, Andree M.; Roy, Kenneth L.; Kan, Yuet Wai
 CS Howard Hughes Med. Inst. Lab., Univ. California, San Francisco, CA, 94143,
 USA
 SO Nature (London, United Kingdom) (1982), 296(5857), 537-40
 CODEN: NATUAS; ISSN: 0028-0836
 DT Journal
 LA English

=> d his

(FILE 'HOME' ENTERED AT 16:42:54 ON 23 OCT 2006)

FILE 'REGISTRY' ENTERED AT 16:43:05 ON 23 OCT 2006

L1 STRUCTURE UPLOADED
 L2 18 S L1 SSS SAM
 L3 2501 S L1 SSS FULL

FILE 'CAPLUS' ENTERED AT 16:44:30 ON 23 OCT 2006

L4 1317 S L3
 L5 1 S L4 AND (NONSENSE(W) SUPPRES?)

FILE 'USPATFULL' ENTERED AT 16:45:11 ON 23 OCT 2006

L6 58 S L3
 L7 0 S L6 AND (NONSNESE(W) SUPPRES?)
 L8 0 S L6 AND (NONSENESE(W) SUPPRES?)
 L9 2 S L6 AND (NONSENSE(W) SUPPRES?)

FILE 'CAPLUS' ENTERED AT 16:45:56 ON 23 OCT 2006

L10 4 S L4 AND ((MUSCULAR(W) DYSTROPHY) OR READTHROUGH OR (CYSTIC(W) FI
 L11 0 S L4 AND (PREMATURE(W) STOP)
 L12 0 S L4 AND (STOP(W) CODON)

INDEX 'ADISCTI, ADISINSIGHT, ADISNEWS, AGRICOLA, ANABSTR, ANTE, AQUALINE,
 AQUASCI, BIOENG, BIOSIS, BIOTECHABS, BIOTECHDS, BIOTECHNO, CABA, CAPLUS,
 CEABA-VTB, CIN, CONFSCI, CROPB, CROPU, DDFB, DDFU, DGENE, DISSABS, DRUGB,
 DRUGMONOG2, DRUGU, EMBAL, EMBASE, ...' ENTERED AT 16:50:08 ON 23 OCT 2006
 SEA (ADENOSINE(W) KINASE) AND ((NONSENSE(W) SUPPRES?) OR READTHRO

 7 FILE GENBANK
 1 FILE PROUSDDR

L13 QUE (ADENOSINE(W) KINASE) AND ((NONSENSE(W) SUPPRES?) OR READTH

FILE 'PROUSDDR' ENTERED AT 16:51:19 ON 23 OCT 2006

L14 1 S (ADENOSINE(W) KINASE) AND ((NONSENSE(W) SUPPRES?) OR READTHROUGH

INDEX 'ADISCTI, ADISINSIGHT, ADISNEWS, AGRICOLA, ANABSTR, ANTE, AQUALINE,
 AQUASCI, BIOENG, BIOSIS, BIOTECHABS, BIOTECHDS, BIOTECHNO, CABA, CAPLUS,
 CEABA-VTB, CIN, CONFSCI, CROPB, CROPU, DDFB, DDFU, DGENE, DISSABS, DRUGB,
 DRUGMONOG2, DRUGU, EMBAL, EMBASE, ...' ENTERED AT 17:10:06 ON 23 OCT 2006
 SEA (GENE(W) THERAPY) AND (AUTOIMMUN? OR ARTHRTIS OR (MULTIPLE(W)

 6 FILE ADISCTI
 52 FILE ADISINSIGHT
 26 FILE ADISNEWS
 7 FILE AGRICOLA
 5 FILE ANTE
 331 FILE BIOENG
 1502 FILE BIOSIS
 7290 FILE BIOTECHABS
 7290 FILE BIOTECHDS
 1426 FILE BIOTECHNO
 19 FILE CABA
 3295 FILE CAPLUS

64 FILE CEABA-VTB
 195 FILE CIN
 27 FILE CONFSCI
 1 FILE CROPU
 362 FILE DDFU
 397280 FILE DGENE
 61 FILE DISSABS
 400 FILE DRUGU
 25 FILE EMBAL
 2232 FILE EMBASE
 938 FILE ESBIODBASE
 1 FILE FROSTI
 53 FILE GENBANK
 421 FILE IFIPAT
 186 FILE IMSDRUGNEWS
 116 FILE IMSRESEARCH
 372 FILE JICST-EPLUS
 460 FILE LIFESCI
 1760 FILE MEDLINE
 15 FILE NTIS
 883 FILE PASCAL
 62 FILE PCTGEN
 43 FILE PHAR
 93 FILE PHARMAML
 1 FILE PHIC
 360 FILE PHIN
 2436 FILE PROMT
 4 FILE PROUSDDR
 1 FILE RDISCLOSURE
 1951 FILE SCISEARCH
 864 FILE TOXCENTER
 14324 FILE USPATFULL
 1527 FILE USPAT2
 5447 FILE WPIDS
 32 FILE WPIFV
 5447 FILE WPINDEK

L15 QUE (GENE(W) THERAPY) AND (AUTOIMMUN? OR ARTHRTIS OR (MULTIPLE(

FILE 'EMBASE, MEDLINE, CAPLUS' ENTERED AT 17:13:44 ON 23 OCT 2006

L16 7287 S (GENE(W)THERAPY) AND (AUTOIMMUN? OR ARTHRTIS OR (MULTIPLE(W)S

L17 0 S L16 AND CLITOCINE

L18 14 S L16 AND (NONSENSE)

=> logoff

ALL L# QUERIES AND ANSWER SETS ARE DELETED AT LOGOFF

LOGOFF? (Y)/N/HOLD:y

COST IN U.S. DOLLARS

SINCE FILE

TOTAL

ENTRY

SESSION

FULL ESTIMATED COST

65.12

357.99

DISCOUNT AMOUNTS (FOR QUALIFYING ACCOUNTS)

SINCE FILE

TOTAL

ENTRY

SESSION

CA SUBSCRIBER PRICE

-6.00

-9.00

STN INTERNATIONAL LOGOFF AT 17:15:08 ON 23 OCT 2006